BEFORE THE

INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE
AND THE APPLICATION REVIEW SUBCOMMITTEE
TO THE
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
ORGANIZED PURSUANT TO THE
CALIFORNIA STEM CELL RESEARCH AND CURES ACT

REGULAR MEETING

LOCATION: SAN FRANCISCO AIRPORT MARRIOTT
WATERFRONT, BAYSIDE ROOM II & III
1800 OLD BAYSHORE HIGHWAY
BURLINGAME, CA 94010

DATE: JANUARY 30, 2025
9 A.M.

REPORTER: BETH C. DRAIN, CA CSR
CSR. NO. 7152

FILE NO.: 2025-2

I N D E X

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I N D E X (CONT'D.)

JANUARY 30, 2025; 9 A.M.

CHAIRMAN IMBASCIANI: GOOD MORNING, LADIES AND GENTLEMEN. I'D LIKE TO WELCOME THE MEMBERS OF THE BOARD AND THE PUBLIC TO THIS, THE 164TH MEETING. OF THE INDEPENDENT CITIZENS OVERSIGHT COMMITTEE, AND THE 60TH MEETING OF THE APPLICATION REVIEW SUBCOMMITTEE OF THE BOARD. I CALL THE MEETING TO ORDER. AND WE WILL STAND AND I WILL LEAD THE PLEDGE OF ALLEGIANCE.

(THE PLEDGE OF ALLEGIANCE.)

CHAIRMAN IMBASCIANI: THANK YOU. I'M GOING TO ASK OUR DIRECTOR OF BOARD GOVERNANCE, SCOTT TOCHER, TO CALL THE ROLL.

MR. TOCHER: ABSOLUTELY. EYAD ALMASRI.

DR. ALMASRI: YES.

MR. TOCHER: KIM BARRETT.

DR. BARRETT: PRESENT.

MR. TOCHER: DAN BERNAL. GEORGE BLUMENTHAL.

DR. BLUMENTHAL: HERE.

MR. TOCHER: MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: PRESENT.

MR. TOCHER: JOHN CARETHERS.

DR. CARETHERS: PRESENT.

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MR. TOCHER: JUDY CHOU.
DR. CHOU: PRESENT.
MR. TOCHER: LEONDRA CLARK-HARVEY.
DR. CLARK-HARVEY: PRESENT.
MR. TOCHER: DEBORAH DEAS.
DR. DEAS: HERE.
MR. TOCHER: ANNE-MARIE DULIEGE.
DR. DULIEGE: HERE.
MR. TOCHER: YSABEL DURON.
MS. DURON: HERE.
MR. TOCHER: MARK FISCHER-COLBRIE.
DR. FISCHER-COLBRIE: HERE.
MR. TOCHER: ELENA FLOWERS.
DR. FLOWERS: PRESENT.
MR. TOCHER: JUDY GASSON. DAVID HIGGINS.
DR. HIGGINS: HERE.
MR. TOCHER: VITO IMBASCIANI.
CHAIRMAN IMBASCIANI: PRESENT.
MR. TOCHER: RICH LAJARA.
MR. LAJARA: PRESENT.
MR. TOCHER: PAT LEVITT.
DR. LEVITT: PRESENT.
MR. TOCHER: HALA MADANAT.
DR. MADANAT: PRESENT.
MR. TOCHER: LINDA MALKAS.

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DR. MALKAS: HERE.
MR. TOCHER: SHLOMO MELMED. CAROLYN MELTZER.
DR. MELTZER: PRESENT.
MR. TOCHER: CHRISTINE MIASKOWSKI.
DR. MIASKOWSKI: PRESENT.
MR. TOCHER: ADRIANA PADILLA.
DR. PADILLA: HERE.
MR. TOCHER: JOE PANETTA.
MR. PANETTA: HERE.
MR. TOCHER: JOYCE SACKEY.
DR. SACKEY: HERE.
MR. TOCHER: MARVIN SOUTHARD.
DR. SOUTHARD: PRESENT.
MR. TOCHER: SUZANNE SANDMEYER. KAROL WATSON. YAEL WYTE.
DR. WYTE: HERE.
MR. TOCHER: KEVIN XU.
DR. XU: HERE.
MR. TOCHER: KEITH YAMAMOTO.
DR. YAMAMOTO: HERE.
MR. TOCHER: GREAT. THANKS VERY MUCH. MR. CHAIR, WE HAVE A QUORUM.
CHAIRMAN IMBASCIANI: THANK YOU, SCOTT. MEMBERS OF THE BOARD, WE HAVE A MANAGEABLY COMPACT

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AGENDA FOR TODAY'S MEETING. AND I WANT TO ACKNOWLEDGE THAT THIS MEETING IS TAKING PLACE AT A TIME OF ENORMOUS CHANGE AT THE FEDERAL LEVEL AFFECTING OF HOW THIS NATION DEALS WITH ISSUES RELATED TO THE HEALTH OF ITS CITIZENS.

THE VICE CHAIR IN HER REPORT WILL REFER TO SOME OF THE CHANGES OR PAUSES THAT AFFECT SCIENTIFIC FUNDING, RESEARCH, CLINICAL TRIALS, AND OTHER GRANTS AND PROGRAMS. BUT I WANT TO START OFF FIRST WITH AN INTRODUCTION OF A NEW BOARD MEMBER, YAEL WYTE.

YAEL IS CURRENTLY THE PROGRAM AND EDUCATION MANAGER OF THE ALZHEIMER'S ASSOCIATION, CALIFORNIA SOUTHLAND CHAPTER, BASED IN CULVER CITY, CALIFORNIA. SHE'S BEEN IN THIS ROLE SINCE 2016, DIRECTING A GREAT MANY PROGRAMS FOR FAMILIES, PATIENTS, AND CAREGIVERS WITHIN THE ALZHEIMER'S COMMUNITY AND IN THE WIDER COMMUNITY TO INCLUDE LEARNING PROGRAMS FOR FIRST RESPONDERS.

I WOULD LIKE TO ASK YAEL, WHO WAS SWORN IN THIS VERY MORNING, TO INTRODUCE HERSELF TO THE BOARD. YAEL.

MS. WYTE: THANK YOU. MORNING, EVERYBODY. I'M THRILLED TO BE HERE AND LOOKING FORWARD TO LEARNING, ABSORBING, AND PARTICIPATING. THAT WAS A GREAT SUMMARY. I'M FROM L.A., BEEN PRETTY MUCH

WORKING WITH ALZHEIMER'S AND DEMENTIA FOR ABOUT 20 PLUS YEARS. MY FAVORITE THING TO DO IS LEAD SUPPORT GROUPS, AND I LOOK FORWARD TO LEARNING MORE. THANK YOU.

CHAIRMAN IMBASCIANI: THANK YOU, YAEL.

VICE CHAIR BONNEVILLE: WELCOME.

CHAIRMAN IMBASCIANI: WELCOME.

I'D LIKE TO REPORT ON SOME RECENT ACTIVITIES. EARLIER THIS MONTH I JOINED THE VICE CHAIR, OUR PRESIDENT, AND OTHER CIRM LEADERS AT THE ANNUAL NUCLEUS FORUM HELD IN MENLO PARK. THE FORUM BRINGS TOGETHER ABOUT 80 LEADERS IN BIOTECHNOLOGY, MANY OF WHOM HAD BEEN ATTENDING THE FORUM FOR YEARS. INVITEES INCLUDED THE FOUNDER AND THE PRESENT, FUTURE, AND SEVERAL PAST PRESIDENTS OF ISSCR, WHO SPONSORED THE GATHERING, AND INCLUDED LEADING ACADEMIC, RESEARCHERS, AND REPRESENTATIVES OF SEVERAL START-UP COMPANIES, SEVERAL OF WHOM HAVE RECEIVED GRANTS FROM CIRM. ALSO IN ATTENDANCE WERE REPRESENTATIVES OF RELEVANT PHARMA COMPANIES AND INVESTORS IN BIOTECHNOLOGY VENTURES.

THE PROGRAM ALLOWED THESE FOUR GROUPS OF PEOPLE TO INTERFACE AND BUILD COMMUNITY AND PERSONAL RELATIONSHIPS. IT STARTED WITH A RECEPTION AND KEYNOTE ADDRESS BY THE FORMER PRESIDENT OF STANFORD

UNIVERSITY, WHICH WAS ENTERTAININGLY MODERATED BY THE DEAN OF HARVARD MEDICAL SCHOOL, DR. GEORGE DALEY. THE THEME OF THE KEYNOTE WAS THE ROLE OF AI IN THE DEVELOPMENT OF NEW THERAPIES.

THE NEXT DAY THERE WERE THREE PANELS, THE FIRST OF WHICH WAS CHAIRED BY ANDREW PLUMP OF TAKEDA PHARMACEUTICAL. IT FEATURED DEVELOPERS AND EXPERTS FROM BIG PHARMA WHO DISCUSSED THE CHALLENGES OF BRINGING CELL AND GENE THERAPIES TO MARKET. THE EMPHASIS, AS EXPRESSED BY MELISSA THOMAS OF ELI LILLY, AND OTHERS, WAS ON THE NEED FOR THESE TO BE TRANSFORMATIONAL AND NOT MERELY INCREMENTAL TO BE VIABLE COMMERCIALLY. THE OTHER PANELISTS WERE CATHERINE PRIEST OF NEURONA THERAPEUTICS; JAY BRADNER OF AMGEN; AND JULIANNE BRUNO OF CRISPR THERAPEUTICS.

OVERALL THERE WAS A SENSE OF OPTIMISM FOR THE FUTURE. THE SICKLE CELL DRUG CASGEVY BY VERTEX THAT USES CRISPR WAS REFERRED TO AS A NOTABLE STEP FORWARD. CHALLENGES FACING THE FIELD INCLUDE DRUG DELIVERY, SUPPLY CHAIN ISSUES, AND SCALE.

NEXT ON THE AGENDA WAS A SERIES OF SCIENTIFIC TALKS ORGANIZED BY PAST ISSCR PRESIDENT, AMANDER CLARK. THEY SPOTLIT HIGHLIGHTS OF RECENT OR ONGOING RESEARCH RANGING FROM BASIC DISCOVERY

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THROUGH CLINICAL TRIALS. WE HEARD FROM RYAN FLYNN, A YOUNG M.D. PH.D. AT HARVARD, WHO IS EXPLORING AND DEFINING CELL SURFACE RNA BIOLOGY. STANFORD'S MICHELLE MONJE GAVE AN OVERVIEW ON THE NEUROSCIENCE OF CANCER AND ITS IMPLICATIONS FOR THE THERAPY OF GLIOMAS. BENJAMIN REUBENOFF REPORTED ON WORK AT HADASSAH HOSPITAL ON PLURIPOTENT STEM CELLS FROM BASIC RESEARCH TO OPHTHALMIC CLINICAL APPLICATION FOR AGE-RELATED MACULAR DEGENERATION. AND FINALLY, LORENZ STUDER REPORTED ON THE COMPLETION OF THE BLUEROCK/BAYER PHASE 1-2 PARKINSON'S THERAPY PROGRAM AND THE NEXT GENERATION CELL THERAPY AND DRUG DISCOVERY PLATFORMS FOR PARKINSON'S DISEASE.

THE LAST PANEL TITLED "FINANCING COMPANIES TODAY" WAS FOCUSED ON FINANCING COMPANIES IN OUR CONTEMPORARY, INCREASINGLY CHALLENGING WORLD. THE PANEL WAS CHAIRED BY VENTURE CAPITALIST AND C-SUITE HABITUE, KNOWN FOR HER 30 PLUS OF BRINGING VALUE TO SHAREHOLDERS, MS. SUSAN SIEGEL. OTHER PARTICIPANTS INCLUDED VIKRAM BAJAJ OF FORESITE CAPITAL; RAJIV KAUL, A DIRECTOR OF FIDELITY'S SELECT BIOTECHNOLOGY FUND; VINEETA AGARWALA OF ANDREESSEN HOROWITZ, WHICH IS A VENTURE CAPITAL FIRM SPECIALIZING IN AI, BIO AND HEALTH, ISSUES AMONG OTHER TECHNOLOGIES; AND KATIE ELLIAS, AN INVESTIGATOR IN LIFE DESIGNS AND

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MEDICAL DEVICES RECENTLY RECRUITED TO SAB'S BIOTHERAPEUTICS BOARD.

THE MAIN TAKEAWAY WAS THAT VENTURE CAPITAL CONTINUES TO SUPPORT PHASE 2 AND PHASE 3 LATER CLINICAL TRIALS, BUT THEY ARE VERY MUCH LESS INVOLVED IN EARLIER STAGE CLINICAL OR OTHER CLASSES OF EARLY RESEARCH.

THE AUDIENCE WAS ENCOURAGED TO HAVE REGULAR AND OPEN CONVERSATIONS WITH THE FINANCING COMMUNITY AND TO CONTINUE TO WORK ON MANUFACTURING AND THE COST OF GOODS.

THE SESSIONS WERE SIGNIFICANT, THE INTERFACE WAS IMPORTANT. IT ALLOWED OUR CIRM REPRESENTATIVES TO NETWORK, CATCH UP, AND BUILD COMMUNITY WITH EXPERTS AND AWARDEES IN PHARMA, CELL AND GENE THERAPY, AND WITH THE INVESTORS.

LET ME END WITH AN UPDATE ON THE SUSTAINABILITY OF CIRM PROJECT. SINCE THE LAST BOARD MEETING IN DECEMBER, I HAVE HAD INTRODUCTORY DISCUSSIONS WITH A NUMBER OF INDIVIDUALS, MOST OF THEM OUTSIDE OF CIRM, WHO HAVE INSIGHT AND/OR FIRSTHAND EXPERIENCE IN ISSUES THAT ANY OF US CONCERNED ABOUT CIRM'S LONG-TERM VIABILITY NEED TO BE AWARE OF.

THESE INDIVIDUALS INCLUDE A FORMER

GOVERNOR OF CALIFORNIA, THE FOUNDING FATHER OF CIRM AND THE PRINCIPAL AUTHOR OF PROPOSITIONS 71 AND 14, THE CAMPAIGN AND LEGAL ADVISOR FOR THOSE SAME PROPOSITIONS, A FORMER BOARD CHAIR, AND A FORMER PRESIDENT AND CEO OF CIRM.

WE DISCUSSED THE THINKING UNDERLYING EACH OF THE HALF DOZEN DIFFERENT POSSIBLE SOLUTIONS FOR A LONG-TERM SOLUTION TO CIRM'S VIABILITY. FOR REFERENCE PURPOSES, AND IN GENERAL TERMS, THESE INCLUDE THE ROLES OF PHILANTHROPY, OF INVESTMENT, OF PUBLIC/PRIVATE PARTNERSHIP, THE FOUNDATION MODEL, THE ROLE OF THE STATE LEGISLATURE, AND THE BALLOT INITIATIVE PROCESS.

I SHOULD HAVE A ROADMAP FOR HOW TO DEVELOP A SUSTAINABILITY PLAN READY FOR COMMENT BY THE MARCH MEETING. NOTICE I SAID A ROADMAP FOR HOW TO DEVELOP, NOT A ROADMAP FOR SUSTAINABILITY BY MARCH IF THE AGENDA OF THAT MEETING IS NOT STRETCHED TOO LONG BY WHAT WILL BE OUR FIRST HEARING OF NEW OR AMENDED CONCEPT PLANS PURSUANT TO OUR EVOLVING STRATEGIC ALLOCATION FRAMEWORK. THANK YOU FOR THAT.

THAT'S THE CHAIR'S REPORT. I'M NOW GOING TO CEDE THE MICROPHONE TO OUR VICE CHAIR MARIA BONNEVILLE FOR HERS.

VICE CHAIR BONNEVILLE: GOOD MORNING. I

HAVE A FEW UPDATES FOR YOU TODAY. I'LL START WITH GOVERNMENT RELATIONS. AND I DON'T WANT TO OVERSTATE THAT I KNOW WHAT'S GOING ON IN DC BECAUSE I'M NOT SURE ANY OF US DO, BUT I CAN GIVE YOU JUST A HEADS-UP ON WHAT WE'VE BEEN HEARING.

AND AS YOU CAN IMAGINE, I AND THE TEAM HAVE SPENT THE BETTER PART OF THE WEEK TRYING TO UNDERSTAND EXACTLY WHAT THE FLURRY OF EXECUTIVE ORDERS AND MEMOS ENCOMPASS. I'VE BEEN IN CONTACT WITH COLLEAGUES AT NIH, FDA, THE UC SYSTEM, OUR POLICY CONSULTANTS IN DC, AND THE GOVERNOR'S OFFICE.

THERE'S A LOT OF CONFUSION AROUND THE MEMO THAT WAS RELEASED BY THE WHITE HOUSE ON MONDAY EVENING, THAT WAS THEN RESCINDED WEDNESDAY MORNING. COMMUNICATIONS FREEZE IS STILL ON, AND THE EXECUTIVE ORDER AROUND DEI IS STILL IN EFFECT.

SOME KEY ISSUES WE'VE ASKED THEM TO MONITOR ONGOING FOR US IN THIS UNCERTAIN TIME: FUNDING CUTS AND RESTRUCTURING OF THE AGENCIES INCLUDING CDC, FDA, NIH, CHANGES IN THE IRA IMPLEMENTATION, CHANGES TO SUBSIDIES AND MEDICAID INCLUDING MY PILOT PROGRAM FOR SICKLE CELL DISEASE, RARE DISEASE HUB, PEDIATRIC PRIORITY REVIEW VOUCHER BECAUSE IT WAS LEFT OUT OF THE CONTINUING RESOLUTION, STEM CELL TOURISM IN GENERAL, AND

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CHANGES IN REGULATION ASSOCIATED WITH LABORATORY DEVELOPED TESTS, WHICH COULD AFFECT NEWBORN SCREENING AND OTHER PROGRAMS.

SO AS YOU KNOW, CIRM WAS BORN OUT OF RESTRICTIONS IMPOSED BY THE FEDERAL GOVERNMENT ON STEM CELL RESEARCH AND IS NOW FORTUNATE TO BE IN A POSITION WHERE WE CAN CONTINUE TO SUPPORT THE FIELD IN THESE TIMES OF UNCERTAINTY. I THINK WE'RE ALL BRACING FOR THE WORST AS THE SITUATION CONTINUES TO UNFOLD. CIRM WILL REMAIN FLEXIBLE AND ADAPTABLE SO THAT WE CAN HELP OUR COLLEAGUES AND REMAIN A DEDICATED SOURCE OF FUNDING. WE DON'T KNOW QUITE YET HOW THIS WILL AFFECT OUR PROGRAMS, AND THE TEAMS ARE PREPARED TO TAKE ALL OF THIS INTO ACCOUNT AS THEY DEVELOP THE NEW CONCEPT PLANS AND BRING THEM TO THE VARIOUS SUBCOMMITTEES AND BOARD.

WE'LL FIND CREATIVE SOLUTIONS WHILE REMAINING TRUE TO OUR CORE VALUES AS AN ORGANIZATION.

SO I KNOW A LOT OF BOARD MEMBERS PROBABLY HAVE COMMENTS ON WHAT'S GOING ON IN DC. SO I JUST WANT TO ALLOW MY COLLEAGUES TO MAKE ANY COMMENTS THAT THEY WISH TO MAKE ON THAT TOPIC. I HAVE OTHER THINGS TO REPORT IN ON THAT ARE HAPPIER. SO I'LL OPEN IT UP NOW IF ANYONE WANTS TO COMMENT.

DR. LEVITT: WE DON'T HAVE ENOUGH TIME.

VICE CHAIR BONNEVILLE: I REALIZE. HOW ABOUT LIMITED COMMENTS? PAT.

DR. LEVITT: YEAH, I MEAN -- YOU KNOW THE REALITY IS THAT THERE'S -- UNPREDICTABILITY IS A GREATER STRESSOR THAN DIRECT NEGLECT OR ABUSE, BELIEVE IT OR NOT, UNPREDICTABILITY. AND THAT'S THE STATE THAT WE'RE LIVING IN. AND FROM A RESEARCHER PERSPECTIVE, AND I'VE HAD ONE OF MY NEW GRANTS FROZEN. AND AT CHILDREN'S HOSPITAL LOS ANGELES, WE HAVE LIKE OVER A HUNDRED GRANT APPLICATIONS THAT WERE SUBMITTED IN THE FALL THAT ARE JUST SITTING THERE. SO IT'S A REALLY TRYING TIME.

AND BECAUSE THERE'S CHANGES THAT OCCUR ALMOST ON THE HOUR IN TERMS OF INTERPRETING WHAT ARE PURPOSELY WRITTEN, VERY, VERY BROAD DIRECTIVES THAT THEY CAN INTERPRET ANY WAY THAT THEY WANT. SO I THINK WE'RE IN A REALLY CHALLENGING STATE.

I THINK CIRM BEING AWARE OF THIS AND UNDERSTANDING THAT THERE'S LIKELY TO BE MORE AND MORE ATTENTION PAID BY THE RESEARCH COMMUNITY, THE BIOMEDICAL RESEARCH COMMUNITY, UNDERSTANDING WHAT CIRM IS GOING TO BE ABLE TO SUPPORT, I THINK, IS REALLY IMPORTANT FOR US TO HAVE A LITTLE BIT OF FORESIGHT. KIND OF LIKE COMING UP WITH WHAT WE DO

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WHEN WE HAVE TO HAVE A PRESS STATEMENT OR SOMETHING. WE SHOULD HAVE TO THINK ABOUT THAT AND NOT NECESSARILY WAIT UNTIL THE TIDAL WAVE HITS. SO THAT'S WHERE WE ARE.

AND FOR THOSE OF US WHO ARE EXPERIENCING THIS DIRECTLY, AND WE HAVE A DEAN OF THE SCHOOL OF MEDICINE WHO HAS HUNDREDS OF FACULTY THAT ARE DEALING WITH THIS. SO IT'S NOT GREAT.

DR. MELTZER: THANKS, PAT. YEAH. I KNOW WE'RE ALL REALLY CONCERNED, ESPECIALLY ABOUT THE IMPACT ON AND THE NECESSITY TO CONTINUE TO DIVERSIFY CLINICAL TRIALS, TO IMPROVE ACCESS OF CARE, THINGS THAT ARE SO FUNDAMENTAL TO CIRM, AND HOW THAT IS GOING TO BE INFLUENCED ON THE FEDERAL LEVEL IN TERMS OF FUNDING. SO LOTS MORE TO FIGURE OUT, BUT IT IS QUITE CONCERNING.

VICE CHAIR BONNEVILLE: JOHN.

DR. CARETHERS: I WILL ADD THE CONFUSION IS PRETTY WIDESPREAD, NOT ONLY AMONGST OUR FACULTY AND THE RESEARCHERS; BUT AS PAT MENTIONED, THE DEFINITIONS ARE CRAZY. I'M AT SAN DIEGO AND EVEN DONORS HAVE CALLED AND SAID, "SHOULD I DONATE? WHAT'S GOING ON? IS THE RESEARCH GOING TO BE ABLE TO CONTINUE?" SO I THINK IT EXTENDS BEYOND JUST THE RESEARCHERS AND THE ADMINISTRATORS.

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WE HAVE HAD FOUR CONTRACTS SPECIFICALLY TARGETED LARGELY BECAUSE THEY SUPPORTED GENDER EQUITY. AND SO THOSE ARE CEASE AND DESIST ORDERS THAT WE HAVE TO CARRY OUT. THERE'S ALSO, WE HAVE A NUMBER OF FACULTY WHO WORK BETWEEN THE UNIVERSITY AND THE VETERANS ADMINISTRATION HOSPITAL. AND THEY ALL GOT THAT MASS EMAIL OFFER TO RESIGN BY SEPTEMBER. AND I'M TELLING MY FACULTY DON'T PUSH THE BUTTON BECAUSE SALARY, BUT I BET YOU SOME OF THE STAFF AND SOME ERRANT PEOPLE WILL DO THAT. SO IT'S JUST CHAOS.

DR. DEAS: YES. I THINK I WAS NEXT. THANK YOU. YES. I DO BELIEVE THAT AS CIRM WE NEED TO BEGIN TO THINK ABOUT OUR FUNDING, HOW WE'RE GOING TO SUPPORT, ET CETERA. AT THE SAME TIME I WOULD CAUTION THAT WE MUST BE INTENTIONAL, STRATEGIC, AND NOT ENGAGE IN ANTICIPATORY OBEDIENCE AND BEGIN TO UNWIND THINGS IN ANTICIPATION.

WE'VE SEEN IN THE LAST FEW DAYS A FLURRY, A PULL-BACK, AND WE SHOULD BE INTENTIONAL, PAUSE, AND THINK THROUGH IT, AND NOT BEGIN TO UNWIND THINGS.

VICE CHAIR BONNEVILLE: I COMPLETELY AGREE. KIM.

DR. BARRETT: AND JUST TO ADD, I

COMPLETELY ENDORSE WHAT PAT SAID, THAT UNCERTAINTY IS FAR MORE DAMAGING THAN JUST BEING TOLD THAT EVERYTHING IS STOPPING. AND WE ARE TRYING TO GET A SENSE OF OUR OVERARCHING LIABILITY AS THE FEDERAL GOVERNMENT TRIES TO PULL THINGS BACK. THE MEMO THAT WAS RESCINDED SUGGESTED THAT THERE MIGHT EVEN BE EFFORTS TO RECOUP MONIES THAT HAVE ALREADY BEEN AWARDED, WHICH OBVIOUSLY WOULD BE HUGELY DAMAGING BECAUSE WE RELY ON THOSE FOR FACULTY AND STAFF AND TRAINEE SALARIES.

AND I AM PARTICULARLY CONCERNED ABOUT THE IMPACT ON EARLY CAREER INVESTIGATORS WHO ARE THE FUTURE OF STEM CELL RESEARCH AND EVERY OTHER TYPE OF BIOMEDICAL RESEARCH. AND I THINK WE HAVE TO MONITOR THAT VERY CAREFULLY. INEVITABLY WE WERE ALREADY RECEIVING A FLOOD OF APPLICATIONS. THE FLOOD WILL NOW BECOME -- WHAT'S WORSE THAN A FLOOD? -- DELUGE.

DR. MALKAS: A WILDFIRE. TRUST ME. BEEN THERE.

MR. TOCHER: YSABEL.

MS. DURON: OKAY. THANK YOU. AS THE PATIENT ADVOCATE HERE, AS A LATINA, AS A MEMBER OF A COMMUNITY THAT'S UNDER ATTACK ON MULTIPLE SIDES, EVEN THOUGH IT DOESN'T PERSONALLY AFFECT ME, AND AS SOMEONE WHO SAT HERE AND LUCKILY HAD COMPANIONS WHO

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AGREE THAT WE NEEDED A DEI POLICY, I THINK THAT -- AND AS SOMEONE WHO'S A COMMUNICATOR BY TRAINING, I THINK, TO YOUR POINTS, WE NEED TO GET OUR MESSAGES TOGETHER.

WE NEED TO ALSO SEND MESSAGES TO OUR COMMUNITIES, TO THE PUBLIC, OUR TAXPAYERS WHO MAKE THIS POSSIBLE, WHAT WE WANT TO DO, HOW WE'RE GOING TO DO IT. THEY NEED TO BE CLEAR THAT WE'RE STILL HERE TO SUPPORT THE SCIENCE, BUT ALSO TO SUPPORT OUR COMMUNITIES TO MAKE SURE THAT THEY CONTINUE TO BE INCLUDED IN ALL OF THESE ADVANCES AND THE THINGS THAT WE'RE TRYING TO GET DONE.

I JUST THINK IT NEEDS TO -- WE NEED TO MAKE SURE THAT WE SEND A MESSAGE THAT WE'RE NOT ABANDONING PEOPLE BECAUSE THAT'S WHAT WE'RE ALL ABOUT IS PEOPLE. AND AS LONG AS I'VE BEEN AROUND, THIS IS ONE OF THE MOST HORRIFIC TIMES THAT I HAVE EVER EXPERIENCED. AND I KNOW, HAVING SPOKEN TO A LOT OF MY COLLEAGUES, I'M SURE YOU ALL ARE TOO.

AND WELCOME TO OUR PUBLIC AND OUR PARENTS. WE EMBRACE YOU AND WE CARE ABOUT YOU AND WE CARE ABOUT THE ISSUES FOR YOUR FAMILIES, YOUR CHILDREN.

WE JUST HAVE TO SORT OF STAY VIGILANT AND STAY ON TOP. WE SHOULD NOT LEAD FROM BEHIND, I BELIEVE. I THINK IT'S NOT ABOUT MAKING TROUBLE, BUT

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IT IS ABOUT STANDING FOR SOMETHING AND NOT BEING COWED BY FEAR OR OPPRESSION BECAUSE I THINK THAT'S WHAT'S WANTED CURRENTLY.

AND SO I'M JUST -- I'M JUST KIND OF ALLOWING MY EMOTIONS TO COME OUT HERE, BUT I DO KNOW, HAVING MET WITH J.T. AND MARIA AND OUR COMMUNICATIONS TEAM, WE DEFINITELY NEED TO TAKE SOME STANDS. AND I INVITE YOU, IN FACT, I INSIST WITH ALL OF OUR RESEARCH LEADERSHIP COMMUNITY THAT AS WE TRY TO GET MESSAGES OUT THERE, YOU PARTICIPATE WITH US IN WRITING SOME OF THOSE TWO-, THREE-PARAGRAPH RESPONSES FOR OUR NEWSLETTERS, FOR OUR WEBSITE, FOR OUR SOCIAL MEDIA JUST SO WE CAN DISPERSE WHAT WE'RE DOING, HOW WE'RE DOING IT, HOW WE INTEND TO KEEP DOING IT TO AS MANY PEOPLE AS WE CAN COMMUNICATE WITH. BUT THEY NEED TO HEAR IT FROM YOU, THE EXPERTS. THEY NEED TO HEAR FROM YOU, THE SCIENTISTS, THAT WE ARE NOT ABANDONING SCIENCE AND WE'RE NOT ABANDONING OUR PUBLIC, AND WE'RE NOT ABANDONING OUR PATIENTS. THANK YOU.

VICE CHAIR BONNEVILLE: THANK YOU, YSABEL.

COUPLE OF OTHER UPDATES. I'LL START WITH THIS ONE. IT SEEMS VERY SWEET GIVEN THE EVENTS OF THE PAST WEEK. BUT IN EARLY JANUARY OUR FORMER CONTACT AT THE WHITE HOUSE REACHED OUT AND ASKED

CIRM TO ATTEND THE BIDEN ADMINISTRATION'S FINAL EVENT FOCUSED ON CELL AND GENE THERAPY. AND ROSA WENT TO REPRESENT CIRM.

THE FOCUS OF THE FORUM WAS EQUITABLE ACCESS TO CELL AND GENE THERAPIES, INCREASING CAPITAL FLOW FOR RARE DISEASES, AND DRIVING INNOVATION IN MANUFACTURING AND DEVELOPMENT. THAT JUST GOES TO SHOW HOW MUCH HAS CHANGED IN THOSE SHORT TWO WEEKS.

SO THANK YOU, ROSA, FOR GOING AND MAKING CONTACTS AND KEEPING THE SPIRIT ALIVE AND CIRM RELEVANT AT THE FEDERAL LEVEL. SO THANK YOU.

I'M GOING TO REPORT OUT ON ACCESS AND AFFORDABILITY WORKING GROUP ACTIVITIES. I'VE BEEN WORKING WITH ROSA, GEOFF, AND EMILY ON AGENDA TOPICS THAT WILL GO TO THE AAWG OVER THE COURSE OF THE NEXT FEW MONTHS. AND TOP OF THE LIST IS GOAL NO. 5 OF OUR STRATEGIC ALLOCATION FRAMEWORK, WHICH IS TO ENSURE THAT EVERY BLA-READY PROGRAM HAS A STRATEGY FOR ACCESS AND AFFORDABILITY. AND THAT INCLUDES CLINICAL TRIAL DESIGN STRATEGIES THAT ADDRESS INFORMATION NEEDS RELATED TO REIMBURSEMENT, VALUE ASSESSMENTS TO GENERATE EVIDENCE NEEDED TO SUPPORT THE REIMBURSEMENT OF APPROVED PRODUCTS BY PAYERS, AND RESOURCES TO SUPPORT PROGRAMS WITH LIMITED

EXPERIENCE WITH ACCESS PLANNING, AND TO ENABLE THEM TO EVALUATE OPTIONS IN THE AREA SUCH AS MANUFACTURING SO THAT THEY ARE MAKING OPTIMAL DECISIONS THAT CAN GET TO MASS PRODUCTION TO DRIVE DOWN COST.

THAT'S A LOT. SO THE AAWG WILL BE FOCUSING ON THAT FOR THE NEXT SEVERAL MONTHS WITH MEETINGS PLANNED OUT THROUGH JUNE.

LASTLY, IN DECEMBER I WENT -- I ALONG WITH MEMBERS OF TEAM ATTENDED A SMALL CONFERENCE IN SAN DIEGO TO STRATEGIZE AROUND PATIENT ACCESS FOR CELL AND GENE THERAPIES. AND IT WAS EXPERTS IN ADVANCED THERAPIES WERE THERE TO DISCUSS NEW INNOVATIONS FOR EXPANDED AND EQUITABLE ACCESS, ESPECIALLY IN THE AREAS OF COST SAVINGS AND REIMBURSEMENT. AND THEN OTHER AREAS THAT SHAPE COSTS AND PATIENT ACCESS IN CELL THERAPIES, INCLUDING MANUFACTURING AND INFRASTRUCTURE, GREATER UTILIZATION OF COMMUNITY HOSPITALS AND CLINIC, CLINICAL DEVELOPMENT, AND OPERATIONS.

WE HELPED SHAPE THAT AGENDA AND TO ENSURE THAT THE RIGHT MIX OF PARTICIPANTS ATTENDED THE MEETING. THE GROUP DISCUSSED POSSIBLE SOLUTIONS FOR A WIDE VARIETY OF HEALTH CHALLENGES FOR MORE COMMON CONDITIONS SUCH AS BLOOD CANCERS TO AUTOIMMUNE

DISEASE AND RARE INHERITED DISORDERS. SPECIFIC IDEAS THAT COULD BENEFIT THE PEOPLE OF CALIFORNIA INCLUDED NEW WAYS TO UTILIZE THE NETWORK OF CIRM-FUNDED ALPHA CLINICS, DRAWING ON CIRM'S SCIENTIFIC PROMINENCE TO ATTRACT NEW PARTNERS FOR LATE STAGE CLINICAL TRIALS, AND BUILDING ON CIRM'S REAL-WORLD KNOWLEDGE TO CREATE RELIABLE, REPEATABLE CONTRACTING FOR CELL AND GENE THERAPIES. THE CONFERENCE REPORT IS FORTHCOMING, AND I'LL SHARE THAT WITH THE BOARD ONCE IT'S READY.

THAT CONCLUDES MY REMARKS.

CHAIRMAN IMBASCIANI: THANK YOU, MARIA. WE'RE GOING TO MOVE TO AGENDA ITEM NO. 6 NEXT, WHICH IS A HAPPY PART OF THE AGENDA WHERE WE PUBLICLY AND FORMALLY ACKNOWLEDGE AND THANK MEMBERS OF THIS BOARD WHO HAVE LEFT THIS BOARD FOR THEIR SERVICE. AND WE HAVE TWO SUCH PEOPLE TODAY.

BUT BEFORE I GET TO THE RESOLUTIONS, I'M SORRY. THERE WAS AN OVERSIGHT AND I TAKE FULL RESPONSIBILITY OF THIS. THERE WAS A SWEARING IN OF ANOTHER BOARD MEMBER SINCE OUR LAST MEETING, AND I WOULD LIKE TO FORMALLY INTRODUCE HIM TO THIS BOARD, SEATED AT THE FRONT TABLE, DR. JOHN CARETHERS.

JOHN IS THE VICE CHANCELLOR FOR HEALTH SCIENCES AT THE UNIVERSITY OF CALIFORNIA AT SAN

DIEGO SINCE JANUARY OF 2023. IN THIS POSITION HE OVERSEES UC SAN DIEGO'S HEALTHCARE SYSTEM, ITS SCHOOL OF MEDICINE, SCHOOL OF PHARMACY, AND SCHOOL OF PUBLIC HEALTH.

HE'S A TRAINED GASTROENTEROLOGIST WITH A LONG HISTORY OF INTEREST IN AND RESEARCH IN HEREDITARY COLON CANCER GENETICS AND COLON CANCER HEALTH DISPARITIES. HE HAS EDITED JOURNALS LIKE THE *GASTROENTEROLOGY JOURNAL* OF HIS SPECIALTY, AND HE'S BEEN CONTINUOUS -- I WONDER IF WE CAN CONTINUE TO SAY THIS NEXT THING GOING INTO THE FUTURE -- CONTINUOUSLY FUNDED BY THE NIH.

JOHN, WOULD YOU LIKE THE OPPORTUNITY TO ADDRESS THE BOARD?

DR. CARETHERS: THANK YOU FOR THE INTRODUCTION, AND IT'S GREAT TO BE HERE. AND I WANT TO SERVE CIRM AND WILLING TO LEARN FROM ALL OF YOU ON THIS BOARD.

I DO HOPE TO SAY ALL OF US CAN CONTINUE TO BE FUNDED BY NIH IN THE FUTURE. AND I'M STILL HOPEFUL DESPITE ALL THE RECENT CHAOS AND DISRUPTIONS BECAUSE WE NEED TO DO, AS DR. BARRETT MENTIONED, FOR OUR YOUNGER GENERATION. THEY ARE OUR FUTURE, THEY ARE THE ONES WHO'S GOING TO KEEP ADVANCING THE KNOWLEDGE FOR CURES, AND SERVE US, AND TAKE CARE OF

ALL OF US AS WELL.

SO IT'S A PLEASURE TO BE HERE, AND THANK YOU, VITO, FOR THAT WONDERFUL INTRODUCTION.

CHAIRMAN IMBASCIANI: WELCOME, JOHN.

SO GOING BACK TO AGENDA ITEM NO. 6, OUR RESOLUTIONS. THE FIRST FORMER BOARD MEMBER I WOULD LIKE TO HONOR WITH A RESOLUTION IS MOHAMED ABOUSALEM WHO'S SITTING IN THE FIRST ROW IN THE BACK. YOU WILL ALL REMEMBER.

LET ME SAY A FEW THINGS ABOUT MOHAMED MYSELF BEFORE I OPEN IT TO THE FLOOR.

MOHAMED WAS NOMINATED TO THE CIRM BOARD BY GOVERNOR NEWSOM. HE JOINED THE ICOC IN MARCH OF 2022. HE WAS TRAINED AS A CIVIL ENGINEER AND HAS SPECIALIZED TRAINING IN SURVEYING ENGINEERING AND AT THE DOCTORAL LEVEL IN GEOMATICS ENGINEERING. IF YOU'RE CURIOUS ABOUT THAT, JUST ASK ME.

HE HELD C-SUITE POSITIONS IN INDUSTRY WHERE, AMONG OTHER ACCOMPLISHMENTS, HE ESTABLISHED A CORPORATE PATENT COMMITTEE. HE MIGRATED OVER TO THE ACADEMY, HOLDING SUCH POSITIONS AS ASSISTANT VICE CHANCELLOR FOR RESEARCH WHERE HE ESTABLISHED THE BIOTECH WET-LAB INCUBATOR FOR UC SANTA CRUZ START-UPS AND OTHER ACCELERATOR INITIATIVES FOR UC ENTREPRENEURS IN SILICON VALLEY.

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HE BECAME THE INAUGURAL VICE PRESIDENT FOR RESEARCH AND INNOVATION AT SAN JOSE STATE UNIVERSITY FROM 2019 TO 2014 WHERE HE SUPPORTED FUNDING FOR MULTIDISCIPLINARY RESEARCH AND CREATED FELLOWSHIPS FOR BOTH STUDENTS AND FACULTY. HIS VISIONARY LEADERSHIP LED TO THE CREATION OF THE SJSU SILICON VALLEY SMALL BUSINESS DEVELOPMENT CENTER.

MOHAMED AND I OVERLAPPED FOR ONLY ONE YEAR, BUT THAT WAS SUFFICIENT TIME TO APPRECIATE THE TALENT AND DEDICATION HE BROUGHT TO CIRM. HE JOINED THE IP AND INDUSTRY SUBCOMMITTEE OF THE BOARD VERY SHORTLY AFTER JOINING THE BOARD ITSELF, AND HE HIT THE GROUND RUNNING, BECOMING CO-CHAIR RATHER QUICKLY, JOINING HIS TALENTS TO ANOTHER ESTEEMED FORMER BOARD MEMBER, STEVE JUELSGAARD.

MOHAMED PLAYED A LARGE ROLE IN DEFINING OUR WARRANT PROGRAM AND AMENDING OUR CO-FUNDING REQUIREMENTS IN TRANSLATIONAL AND CLINICAL STAGE RESEARCH, BOTH OF WHICH WERE PASSED BY THIS BOARD. HE ALSO SERVED ON THE PRESIDENTIAL SEARCH COMMITTEE THAT LED TO THE HIRING OF OUR FIFTH PRESIDENT AND CEO, DR. JAMES THOMAS.

MOHAMED DREW FROM HIS BROAD, REAL-WORLD EXPERIENCES, WAS ALWAYS READILY AVAILABLE, GAVE VALUABLE FEEDBACK, TESTED ASSUMPTIONS BY INTELLIGENT

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QUESTIONING, AND WAS GENERALLY A JOY TO WORK WITH. HE MADE RECOMMENDATIONS ON DATA SCIENCE TRAINING FOR OUR EDUCATIONAL PROGRAM TRAINEES.

CIRM'S LOSS, MOHAMED, HAS BECOME THE KECK GRADUATE INSTITUTE'S INESTIMABLE GAIN AS HE NOW SERVES AS ITS THIRD PRESIDENT. MOHAMED, COME FORWARD PLEASE. WE HAVE A RESOLUTION FOR YOU. COME, YES.

(APPLAUSE.)

CHAIRMAN IMBASCIANI: OTHER BOARD MEMBERS WOULD LIKE TO SPEAK.

VICE CHAIR BONNEVILLE: LEONDRA.

DR. CLARK-HARVEY: MOHAMED, I WANT TO LOOK AT YOU, BUT IT'S HARD. OUR TIME ON THE BOARD OVERLAPPED JUST A LITTLE BIT, BUT I THINK THAT QUICKLY I GOT A SENSE OF YOUR CHARACTER AND WHO YOU ARE. AND SO I JUST WANTED TO SHARE MY APPRECIATION FOR YOU SHARING THAT WITH US. I ALWAYS LOOKED AT YOU AS A BRAVE VOICE ON THE BOARD. THERE ARE MANY, AND YOU ARE ONE OF THEM. AND YOU ALWAYS REMINDED US WHAT IS FAIR, EQUITABLE AND, RIGHT. AND SO THANK YOU FOR DOING THAT. I KNOW THIS ISN'T THE ONLY SPACE THAT YOU PROBABLY DO THAT IN SPEAKING THAT WAY, BUT THANK YOU FOR DOING IT HERE. IT WAS SO IMPORTANT AND IT WAS NOTICED.

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VICE CHAIR BONNEVILLE: GEORGE.

DR. BLUMENTHAL: WELL, I HAD THE DISTINCT PLEASURE OF WORKING WITH MOHAMED FOR A NUMBER OF YEARS WHEN HE WAS AT UC SANTA CRUZ WORKING IN THE OFFICE OF RESEARCH. AND I CAN SAY THAT WAS A TREMENDOUS PLEASURE FOR ME. AND THAT PLEASURE WAS MULTIPLIED MANY TIMES WHEN I FOUND THAT MOHAMED WAS JOINING THIS BOARD.

HE'S BEEN A VOICE OF SANITY AND REASON AND LOGIC EVER SINCE HE JOINED THE BOARD HERE. AND I REALLY APPRECIATED YOUR PRESENCE. AND, MOHAMED, I, FOR ONE, AM GOING TO MISS YOU A GREAT DEAL, AND I SUSPECT THE REST OF US WILL AS WELL.

VICE CHAIR BONNEVILLE: YSABEL.

MS. DURON: THANK YOU. I'M REALLY GOING TO MISS YOU. I APPRECIATED YOU COMING A BOARD, BUT AS A SAN JOSE STATE GRADUATE, I'M A LITTLE TICKED OFF AT YOU FOR LEAVING US SINCE YOU WERE HELPING CREATE SUCH A WONDERFUL SCIENCE PROGRAM AT SAN JOSE STATE AND ACTUALLY PUSHING IT UP INTO THE ATMOSPHERE TO REALLY MAKE IT A GREAT PLACE TO LEARN AND PREPARE STUDENTS FOR SCIENCE.

SO I WILL KIND OF FORGIVE YOU, BUT I'M SORRY TO SEE YOU GO ALSO FROM THE BOARD. THANK YOU VERY MUCH, AND I HOPE WE CONTINUE TO SEE EACH OTHER

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SOME WAY.

VICE CHAIR BONNEVILLE: PAT AND THEN HALA.

DR. LEVITT: WELL, I WAS -- I'M GOING THE SAME DIRECTION. I'M GOING TO PILE ON WHAT YOU JUST SAID. I KNOW THE KECK INSTITUTE HAS, WILL BENEFIT GREATLY FROM ALL OF YOUR EXPERTISE AND YOUR HUMANITY, WHICH CAME THROUGH IN YOUR SERVICE ON THE BOARD, BUT I'M NOT HAPPY THAT YOU HAVE LEFT THE BOARD. I CAME A LITTLE BIT BEFORE YOU, AND YOU'VE MADE A HUGE DIFFERENCE -- YOU MADE A HUGE DIFFERENCE IN A VERY SHORT PERIOD OF TIME. SO CONGRATULATIONS TO THE KECK INSTITUTE. I FEEL BADLY FOR SAN DIEGO STATE --

DR. BARRETT: SAN JOSE STATE.

DR. LEVITT: SAN JOSE STATE. SORRY. SORRY. MY BRAIN IS IN SAN DIEGO WHERE I'M AN ALUMNUS. SAN JOSE STATE. THIS IS WELL DESERVED.

DR. MADANAT: SO MOHAMED AND I DIDN'T OVERLAP AT CIRM. ACTUALLY I TOOK KIND OF HIS SPOT WHEN HE LEFT. SO LITTLE DIFFERENT EXPERIENCE HERE. BUT MOHAMED AND I, WHEN I JOINED MY ROLE AS VICE PRESIDENT, HE WAS THE OTHER VICE PRESIDENT AT SAN JOSE STATE, AND VERY FEW OTHERS IN THE SYSTEM, IT WAS ONE MORE ONLY WITHIN THE CSU. AND WITHOUT HIM, I COULDN'T HAVE DONE MY JOB AT SAN DIEGO STATE,

LEARNED INCREDIBLY A LOT FROM HIM. AND I MISS HIM IN THAT ROLE AS A COUNTERPART WITHIN THE SYSTEM, AND I'M SURE ALL OF YOU DO HERE BECAUSE WHAT I LEARNED FROM HIM TRANSLATES IN EVERY SETTING THAT I'VE SEEN HIM IN. THANK YOU FOR ALL YOU DO.

VICE CHAIR BONNEVILLE: SO THE GOVERNOR IS THE ONLY ONE WHO CAN APPOINT SOMEONE FROM THE CAL STATE SYSTEM. AND SO HE MADE AN AMAZING CHOICE. AND SO I THANK HIM, BUT I THANK YOU FOR BEING ON THE BOARD. I'M REALLY LUCKY. I'VE GOT -- I'VE HAD A CHANCE TO WORK WITH THE BOARD AND BE PART OF THE BOARD FOR ALL OF THESE YEARS. AND I GET TO SPEND TIME WITH YOU, AND IT WAS REALLY WONDERFUL.

YOU WERE ALWAYS VERY REASONABLE WHEN I SOMETIMES CALLED SOUNDING UNREASONABLE. AND I REALLY APPRECIATED JUST THAT COUNTERPOINT AND THAT UNDERSTANDING OF, YOU KNOW, IF YOU LOOK AT IT THIS WAY, MARIA. OKAY. I'LL LOOK AT IT THAT WAY. SO THANK YOU.

CHAIRMAN IMBASCIANI: J.T.

DR. THOMAS: SO, MOHAMED, WHEN -- FIRST OF ALL, WHEN I WAS READING YOUR RESOLUTION, AND THIS SORT OF APPLIES VERY MUCH TO MOHAMED, BUT TO EVERYBODY HERE, AND YOU READ DOWN EVERYTHING YOU'VE DONE. AND WE ALL SORT OF SIT HERE AND VIEW

OURSELVES IN THE CONTEXT OF CIRM AND SORT OF FORGET ABOUT EVERYTHING THAT CAME BEFORE THAT. THE LITANY OF THINGS THAT YOU'VE DONE WAS UNBELIEVABLE AND SO IMPRESSIVE AND, I'M SURE, CENTRAL TO THE GOVERNOR APPOINTING YOU TO THE BOARD. AND I INVITE PEOPLE TO READ MOHAMED'S RESOLUTION. REALLY AMAZING.

SECONDLY, WHEN YOU JOINED THE BOARD AT A TIME WHEN I WAS SORT IN THE TAIL END OF MY TENURE AS BOARD CHAIR, YOU CAN TELL VERY QUICKLY HOW PARTICIPATORY AND INTERESTED IN CERTAIN THINGS, DIFFERENT BOARD MEMBERS -- EVERYBODY COMES WITH DIFFERENT SETS OF INTEREST. AND SEEING WHERE YOU WERE COMING FROM AND THE SORTS OF THINGS THAT YOU EMPHASIZED WANTED TO HAVE YOU ON THE IP AND INDUSTRY SUBCOMMITTEE. AND AS YOU WILL RECALL, ONE DAY YOU WOKE UP AND I HAD APPOINTED YOU AS CO-CHAIR. AND YOU SAID, "THAT'S VERY INTERESTING. THANK YOU VERY MUCH." I SAID, "WELL, THE REASON THAT I DID THAT WAS THAT YOU ARE SO COMPETENT IN THAT SPACE AS WELL AS EVERYTHING ELSE, THAT IT WAS A ROLE THAT WAS PERFECTLY SUITED FOR YOU." AND, OF COURSE, AT THE TIME ECHOING COMMENTS WE WERE HOPING YOU WOULD SERVE AS FOR MANY YEARS, BUT AS WE KNOW NOW, YOU'VE GONE ON TO OTHER THINGS.

BUT I JUST WANTED TO SAY WHAT A PLEASURE

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IT WAS HAVING YOU HERE. YOU WERE PART OF THIS ILLUSTRIOUS BODY WHERE EVERYBODY CONTRIBUTES SO MUCH. YOU BROUGHT SPECIFIC EXPERTISE THAT NOBODY ELSE DID, ALL OF WHICH WAS SO HELPFUL. SO THANK YOU AND WELL DESERVED NEW POSITION THAT YOU HAVE, AND THEIR GOOD FORTUNE IS OUR LOSS, BUT WE APPLAUD YOU FOR EVERYTHING. SO THANK YOU.

DR. ABOUSALEM: THANK YOU VERY MUCH.

(APPLAUSE.)

VICE CHAIR BONNEVILLE: WE NEED A MOTION TO ACCEPT THE RESOLUTION -- ADOPT THE RESOLUTION.

DR. BLUMENTHAL: SO MOVED.

DR. SOUTHARD: SECOND.

MR. TOCHER: GEORGE, SECOND BY MARV.

VICE CHAIR BONNEVILLE: SCOTT, WILL YOU TAKE THE ROLL.

MR. TOCHER: ALL THOSE IN THE ROOM IN FAVOR SAY AYE. THOSE OPPOSED? ANY ABSTENTIONS? I'LL POLL THE MEMBERS ON THE PHONE.

JUDY CHOU.

DR. CHOU: AYE.

MR. TOCHER: DAVID HIGGINS.

DR. HIGGINS: YES.

MR. TOCHER: RICH LAJARA.

MR. LAJARA: YES.

MR. TOCHER: ADRIANA PADILLA.
DR. PADILLA: YES.
MR. TOCHER: JOE PANETTA.
MR. PANETTA: AYE.
MR. TOCHER: KEVIN XU.
DR. XU: AYE.
MR. TOCHER: AND KEITH YAMAMOTO.
DR. YAMAMOTO: AYE.
MR. TOCHER: THANK YOU. THE MOTION IS ADOPTED.
CHAIRMAN IMBASCIANI: THANK YOU, MOHAMED.
DR. ABOUSALEM: THANK YOU SO MUCH. JUST A FEW WORDS. I KNOW YOU HAVE A PACKED AGENDA. BUT GOOD MORNING TO ALL OF YOU, MR. CHAIR, MADAM VICE CHAIR, MR. PRESIDENT, ENTIRE ICOC GOVERNING BOARD, AND ALL CIRM STAFF. IT IS TRULY AN HONOR TO STAND BEFORE YOU TODAY.
DURING MY TENURE ON THE CIRM GOVERNING BOARD, I WAS PRIVILEGED TO SERVE ALONGSIDE ITS TEAM, SCIENCE AND COMMUNITY LEADERS WHO SHARED THE UNWAVERING COMMITMENT TO HEALING AND UPLIFTING OUR COMMUNITIES THROUGH ADVANCED TREATMENTS AND CURES FOR CHRONIC DISEASES.
I WAS PARTICULARLY IMPRESSED BY THE DEDICATION AND PASSION DISPLAYED BY ALL BOARD

MEMBERS IN EVERY DECISION REGARDLESS OF ITS MAGNITUDE AND THE TRUE COMMITMENT TO ALL PATIENTS, INCLUDING THOSE FROM UNDERREPRESENTED AND HISTORICALLY UNDERSERVED COMMUNITIES.

THE CIRM OPERATION IS TRULY A TESTAMENT TO THE PROFESSIONALISM AND CARE OF ITS STAFF WHO DILIGENTLY AND EQUITABLY INVEST IN PROGRAMS THAT DELIVER EXCEPTIONAL RESULTS. WHILE THE SCIENTIFIC RESEARCH AND DEVELOPMENT SUPPORTED BY CIRM IS NOT DIRECTLY PERFORMED ON-SITE, THE ORGANIZATION'S SOPHISTICATED AND ENTREPRENEURIAL APPROACH SETS IT AS A GOLD STANDARD FOR GRANTING PROGRAMS SUPPORTING HEALTH SCIENCES.

I AM PROUD TO HAVE BEEN A PART OF THIS WORLD-CLASS OPERATION DEDICATED TO THIS WORTHY CAUSE. I EXTEND MY SINCERE GRATITUDE TO YOUR RECOGNITION TODAY. A SPECIAL THANK-YOU GOES TO GOVERNOR NEWSOM FOR HAVING ENTRUSTED ME WITH THIS IMPORTANT RESPONSIBILITY. I HOPE THAT MY CONTRIBUTIONS HAVE IN SOME WAY ADVANCED THE VALUE OF THE INVALUABLE WORK THAT YOU ALL DO. THANK YOU VERY MUCH.

(APPLAUSE.)

CHAIRMAN IMBASCIANI: I HAVE A SECOND RESOLUTION. THIS IS WITH REGARD TO AL ROWLETT WHO

IS NOT WITH US HERE IN THE ROOM, BUT HE IS ONLINE. HE'S ON ZOOM. GOOD. WELCOME, AL.

AL ROWLETT JOINED THE CIRM BOARD OF DIRECTORS IN 2013 AT THE RECOMMENDATION OF THE SPEAKER OF THE CALIFORNIA ASSEMBLY TO SERVE AS THE MENTAL HEALTH PATIENT ADVOCATE. DURING HIS 11 YEARS WITH US, HE SERVED AS CHAIR OF THE FINANCE SUBCOMMITTEE, A MEMBER OF THE APPLICATION REVIEW AND GOVERNANCE SUBCOMMITTEE, THE TASK FORCE ON NEUROSCIENCE AND MEDICINE, THE GRANTS WORKING GROUP, AND THE ACCESS AND AFFORDABILITY WORKING GROUP. HE WORKED TIRELESSLY IN OPERATIONALIZING CIRM'S COMMITMENT TO DIVERSITY, EQUITY, AND INCLUSION.

HE'S MADE A PROFOUND IMPACT ON THE STATE, BUILDING ON HIS FORMAL TRAINING IN SOCIAL WORK AND BUSINESS ADMINISTRATION. THIS LED HIM IN 1981 TO TURNING POINT COMMUNITY PROGRAMS ACTIVE IN MENTAL HEALTH AND COMMUNITY SUPPORT, RAISING OVER 43 YEARS FROM REHABILITATION -- RISING OVER 43 YEARS FROM REHABILITATION COUNSELOR TO CHIEF EXECUTIVE OFFICER.

AL EXPANDED TCPC'S IMPACT THROUGHOUT NINE NORTHERN CALIFORNIA COUNTIES BY EMPHASIZING THE BENEFIT OF HOUSING AND RESPITE SERVICES AS AN ALTERNATIVE TO EMERGENCY ROOM VISITS OR PSYCHIATRIC HOSPITALIZATIONS. HE SERVED AS THE BOARD CHAIR OF

THE CALIFORNIA INSTITUTE OF BEHAVORIAL HEALTH SOLUTIONS, A BOARD DIRECTOR OF THE UNITED STATES PSYCHIATRIC REHABILITATION ASSOCIATION, AND AS COMMISSIONER OF THE CALIFORNIA MENTAL HEALTH SERVICES OVERSIGHT AND ACCOUNTABILITY COMMISSION, WHICH CAME ABOUT AFTER THE PASSAGE OF THE MENTAL HEALTH BALLOT INITIATIVE KNOWN AS PROPOSITION 1.

A MAN OF FAITH, HE ROUNDS OUT ALL OF THIS WORK OF SERVICE AS THE BOARD CHAIR OF THE FELLOWSHIP OF CHRISTIAN ATHLETES IN SACRAMENTO. AL CONTINUES TO BE AN ACTIVE FINANCIAL STEWARD OF CIRM THROUGH HIS SERVICE NOW ON THE CITIZENS FINANCIAL ACCOUNTABILITY OVERSIGHT COMMITTEE. THIS IS A SIX-MEMBER BOARD CHAIRED BY THE STATE CONTROLLER THAT EACH YEAR REVIEWS CIRM'S FINANCIAL PRACTICES AND PERFORMANCE. IN FACT, J.T. WILL REPORT ON THIS MOST RECENT MEETING OF THE CFAOC WHERE WE HAD A SWEET REUNION WITH AL ROWLETT.

I AM LEAVING AL'S OTHER ACTIVITIES, INCLUDING HIS OUTREACH -- I'M LEAVING OUT HIS OTHER ACTIVITIES, INCLUDING HIS OUTREACH TO THE HOMELESS IN SAN FRANCISCO AND SACRAMENTO AND HIS MISSION TRIPS TO ORPHANAGES OR TO BUILD HOMES OR TO CREATE WATER ACCESS IN GHANA, SPAIN, JAMAICA, AND MEXICO.

AL, WE DON'T KNOW HOW YOU MANAGE TO

SQUEEZE US INTO THIS BUSY LIFE OF YOURS. WE CERTAINLY THANK YOU FOR YOUR SERVICE. WE WELCOME YOU HERE TODAY TO HEAR OUR COMMENTS FOR YOU.

ARE THERE ANY BOARD MEMBERS? YES. I'LL START WITH MARV.

DR. SOUTHARD: SO, AL, I THINK CIRM WAS THE FIFTH BOARD THAT WE SERVED ON TOGETHER. AND I KIND OF THINK, AL, EVEN THOUGH YOU'RE YOUNGER THAN I AM, I THINK OF YOU AS MY OLDER BROTHER BECAUSE YOU'VE SHOWN ME HOW TO DO THIS WORK SO CLEARLY AND DEFINITELY.

AS WE FIRST STARTED DOING THE DEI REVIEWS, THERE WERE TIMES WHEN SOME OF THE REVIEWS WERE MINIMAL, I THINK, WOULD BE THE WORD. AND YOU WOULDN'T PUT UP WITH THAT. AND ONE OF THE THINGS THAT WE LEARNED HOW TO DO IS TO DO A REAL, HONEST, AND TRUE DEI EVALUATION OF THE PROPOSALS. AND ONE OF THE THINGS THAT'S HAPPENED OVER TIME, THEY'RE INFINITELY BETTER THAN THEY WERE IN THE EARLY STAGES. IT REALLY IS TRUE. AND, AL, YOU MADE THAT HAPPEN.

I THINK FOR ME THE MOST IMPORTANT THING THOUGH, AL, IS THE WORK THAT YOU HAVE DONE IN TURNING POINT WAS A REAL, TRUE COMMUNITY SERVICE. YOU PUT THE DEI IDEAS INTO ACTUAL PRACTICE IN REAL

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COMMUNITIES. AND AS TURNING POINT HAS FLOURISHED BECAUSE OF YOUR LEADERSHIP, IT WAS REALLY REMARKABLE TO SEE DEI AND COMMUNITY OUTREACH REALLY, HONEST TO GOD HAPPEN INSTEAD OF JUST BEING PRO FORMA.

SO, AL, YOU HAVE BEEN A LEADER. NOW, AS YOU'VE RETIRED, WHAT, TWO DAYS AGO FROM TURNING POINT, TURNING POINT IS AT A TURNING POINT. BUT YOU WILL CONTINUE TO SERVE AS YOU ALWAYS HAVE, AND YOUR FAITH IS REALLY EVIDENT, AND IT NOURISHES AND GUIDES US ALL. SO, AL, AS YOUR YOUNGER BROTHER, THANK YOU.

VICE CHAIR BONNEVILLE: I THINK LEONDRA WAS NEXT AND THEN CHRIS AND KIM.

DR. CLARK-HARVEY: I HAD TO WRITE THIS DOWN SO I DON'T GET TEARFUL. I THINK IT'S REALLY FITTING ON THE HEELS OF MLK'S BIRTHDAY A FEW WEEKS AGO AS WELL AS A FEW DAYS AWAY FROM STEPPING INTO BLACK HISTORY MONTH TO SHARE THIS QUOTE FROM MARTIN LUTHER KING THAT MAKES ME THINK OF AL. AND THAT IS "OF ALL THE FORMS OF INEQUALITY, INJUSTICE, HEALTHCARE IS THE MOST SHOCKING AND INHUMANE." AND THOUGH THAT'S VERY SUMMERATE, IT STILL RINGS TRUE TODAY. AND I THINK IT IS FITTING FOR YOU, AL, BECAUSE YOU REALLY HAVE DEDICATED YOUR LIFE, YOUR CAREER PERSONALLY AND PROFESSIONALLY TO AMELIORATING HEALTH DISPARITIES, PARTICULARLY BEHAVORIAL HEALTH

DISPARITIES, AND YOU'VE DONE THIS THROUGH YOUR PERSONAL LIFE. YOU'VE DONE THIS THROUGH YOUR COMMUNITY LEADERSHIP AND ALL OF THE ACCOLADES. AND YOU'VE DONE THIS THROUGH YOUR SERVICE AT CIRM AS WELL.

SO THANK YOU FOR BEING A PERSONAL FRIEND AND A MENTOR AND A LEADER. AND I WILL SEE YOU BECAUSE YOU STILL SIT ON 50 MILLION BOARDS. I JOKE WITH AL THAT HE'S NOT REALLY RETIRING. I DON'T THINK HE KNOWS HOW TO. BUT THANK YOU. THANK YOU FOR THE LEADERSHIP, THANK YOU FOR THE CARE FOR YOUR FAMILY, YOUR COMMUNITY, AND THANK YOU FOR EVERYTHING YOU WILL CONTINUE TO DO FOR THE BEHAVORIAL HEALTH COMMUNITY.

DR. MIASKOWSKI: AL, I WANT TO ECHO MARV'S ELOQUENT COMMENTS AND EXPRESS TO YOU MY GRATITUDE. YOU MAY NOT KNOW THIS, BUT COMING ON THE BOARD IS AN OVERWHELMING EXPERIENCE. AND I LEARNED SO MUCH FROM YOU PARTICULARLY IN THE GWG IN TERMS OF HOW TO WRITE DEI REVIEWS, HOW TO SPEAK UP AND ADVOCATE FOR PATIENTS. I WAS VERY, VERY, VERY SAD WHEN THEY SAID YOU WERE STEPPING DOWN FROM THE BOARD. YOU ARE TRULY MISSED. AND I JUST WANT TO THANK YOU SO, SO MUCH FOR ALL YOU DID AND ARE CONTINUING TO DO.

CHAIRMAN IMBASCIANI: THANK YOU,

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CHRISTINE.

DR. BARRETT: AL, YOU MAY NOT KNOW THE IMPACT THAT YOU'VE HAD ON A LOT OF PEOPLE, AND YOU CERTAINLY PROBABLY DON'T KNOW THE IMPACT THAT YOU'VE HAD ON ME. BUT IN MUCH THE SAME WAY THAT CHRIS SAID, THAT COMING ON THIS BOARD IS A LITTLE OVERWHELMING AND INTIMIDATING, ONE OF THE EARLY TOUCHPOINTS FOR ME WAS TO WATCH HOW YOU CONDUCTED YOURSELF AND HOW YOU ALWAYS HAD SOMETHING IMPORTANT TO SAY WHEN YOU COMMENTED ON ANY ISSUE.

I WOULD SAY, AND I GUESS IT'S CONSISTENT WITH EVERYTHING THAT WE'VE HEARD ABOUT YOUR LIFE AND YOUR CONTRIBUTIONS AND YOUR SERVICE, THAT YOU WERE A MORAL COMPASS FOR THIS BOARD. AND I PERSONALLY REALLY APPRECIATED THAT. SO WE DIDN'T HAVE THE OPPORTUNITY TO GET TO KNOW EACH OTHER VERY WELL PERSONALLY, BUT KNOW THAT THERE ARE PEOPLE OUT THERE WATCHING YOU THAT YOU MAY NEVER KNOW THAT YOU INFLUENCED, BUT YOU REALLY HAVE. SO THANK YOU SO MUCH, AND I TOO VERY MUCH MISS HAVING YOU ON THIS BOARD WITH US.

CHAIRMAN IMBASCIANI: THANK YOU. YSABEL.

MS. DURON: AL, I MISS YOU. I MISS YOUR QUIET BUT FIRM, COMMANDING PRESENCE. AND AS KIM SAID, I MISS THE COMMANDING WAY IN WHICH YOU HANDLE

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THE TOPICS. I MISS FEELING LIKE SOMEONE'S GOT MY BACK, AND I APPRECIATE THAT YOU WERE HERE AND HOLDING DOWN THE FORT AND MAKING SURE DEI IS PRESENT AND THAT IT BECAME STRONGER AND VERY MUCH A PART OF CULTURE AND HOW WE LOOK AT THINGS TODAY. SO I APPRECIATE THAT, BUT I DO MISS YOU, AND I HOPE ALL IS WELL FOR YOU. AND I KNOW THAT YOU WILL CONTINUE TO MAKE GOOD AND BE GOOD OUT THERE IN THE WORLD. THANK YOU.

VICE CHAIR BONNEVILLE: I STARTED WORKING WITH AL THE DAY HE BECAME A BOARD MEMBER, AND I REMEMBER THE FIRST TIME WE MET AND J.T. AND I WENT TO GO VISIT HIM IN HIS OFFICE IN SACRAMENTO. AND I THOUGHT HE IS SUCH A PLEASURE, AND IT WAS SUCH A WELCOMING VOICE ON THE BOARD. AND OVER THOSE 13 YEARS, AL, WE'VE BECOME FRIENDS WHICH TO ME IS VALUABLE AND AMAZING. AND I FEEL REALLY FORTUNATE THAT I'VE HAD THAT OPPORTUNITY AND THAT YOU'VE ALLOWED ME TO BECOME ONE OF YOUR FRIENDS.

AND WORKING WITH YOU AND SITTING ON THE GWG WITH YOU HAS BEEN REALLY REMARKABLE BECAUSE YOU REALLY CHALLENGED ME TO SORT OF LOOK AT THINGS IN A DIFFERENT PERSPECTIVE AND SEE THINGS THAT I MISSED THE FIRST TIME AROUND AND REALLY CHALLENGED PERHAPS MY THOUGHTS AROUND WHAT A GOOD DEI PLAN MIGHT BE.

SO THANK YOU SO MUCH FOR EVERYTHING. AND I KNOW THAT IF I ASK YOU TO DO SOMETHING, YOU'LL DO IT. SO BE CAREFUL. YOU MAY BE SEEING MORE OF US. SO THANK YOU FOR EVERYTHING YOU'VE DONE FOR US.

CHAIRMAN IMBASCIANI: THANK YOU, MARIA. J.T.

DR. THOMAS: AL, SO YOU ARE AND ALWAYS WILL BE THE MAN. CHAIRS OF BOARDS ALWAYS HAVE GO-TO PEOPLE THAT THEY PARTICULARLY RELY ON FOR THE SOUNDEST ADVICE. AND YOU AND I OVER OUR MANY YEARS TOGETHER HAD COUNTLESS DISCUSSIONS, NOT JUST IN THE CONTEXT OF SUBCOMMITTEES WHICH YOU EITHER CHAIRED OR WERE A MEMBER OF, BUT ABOUT ALL THINGS CIRM. AND YOU UNFAILINGLY GAVE OUTSTANDING ADVICE, WHICH I TOOK TO HEART IN EACH AND EVERY INSTANCE. AND THAT HELPED ME AND THE BOARD ADVANCE IN A WAY THAT CONTINUED TO GIVE US OUR BEST SHOT AT FULFILLING OUR MISSION.

YOU WERE A UNIQUE VOICE ON THE BOARD. YOUR COMBINATION OF EXPERTISE, OF FAITH, OF CARING, OF WANTING TO DO WHAT WAS THE RIGHT THING AT ALL TIMES MADE A HUGE DIFFERENCE. WHEN WE HAD THE OPPORTUNITY TO ADD SOME NEW MEMBERS OF THE BOARD IN THE MENTAL HEALTH AREA, YOU WILL RECALL, AS I DO, VIVIDLY OUR DISCUSSION IN WHICH CASE -- WHICH TIME

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YOU SAID, "YOU KNOW, I'VE GOT THESE TWO FRIENDS NAMED LEONDRA AND MARV." AND THAT SENTENCE LED TO THE APPOINTMENT AND JOINING OF TWO MARVELOUS BOARD MEMBERS WHO BROUGHT SO MUCH WHICH YOU KNEW THEY WOULD.

AND JUST WANT TO SAY THAT WE REALLY MISS YOU, REALLY, ALL CAPS, AND FEEL PRIVILEGED TO HAVE HAD THE OPPORTUNITY TO WORK WITH YOU FOR SO MANY YEARS. AND, OF COURSE, NO COMMENT WOULD BE COMPLETE WITHOUT SAYING THAT YOU SENDING ME ALL THOSE TEXTS ABOUT THE GIANTS WERE KIND OF IRRITATING, BUT AT THE MOMENT I'VE GOT THE LAST LAUGH. SO I JUST WANT TO SAY THANK YOU FOR ALL YOU DID. IT WAS WONDERFUL SERVING WITH YOU. IT'S WONDERFUL THAT YOU'RE THERE TO GIVE TREMENDOUS CONTEXT AND INSTITUTIONAL MEMORY TO THE CFAOC AS THEY OVERSEE WHAT WE'RE DOING. AND I COUNT YOU AS A GREAT FRIEND AND LOOK FORWARD TO MANY MORE DISCUSSIONS ON MANY TOPICS. SO THANK YOU, AL.

CHAIRMAN IMBASCIANI: THANK YOU, J.T. I'D LIKE TO -- SORRY, ANNE-MARIE.

DR. DULIEGE: JUST SIMPLY TO SAY TO BOTH MOHAMED AND AL, NOTHING ORIGINAL TO ADD TO WHAT WAS SAID, BUT HOW MUCH WE ALREADY MISS YOU. YOU HAD SUCH A PRESENCE AND YOU'RE ALREADY MISSED, BOTH OF

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YOU. SO THANK YOU, AND HOPEFULLY WE'LL ALL STAY IN TOUCH ONE WAY OR ANOTHER. THANK YOU.

CHAIRMAN IMBASCIANI: HAVE I MISSED ANY HANDS? IF NOT, I WOULD LIKE TO CAP OFF THIS CONVERSATION WITH A MOTION TO ACCEPT THIS RESOLUTION IN HONOR OF AL ROWLETT'S SERVICE TO THIS BOARD.

DR. SOUTHARD: SO MOVED.

DR. BARRETT: SECOND.

MR. TOCHER: I DIDN'T HEAR A SECOND.

DR. BARRETT: SECOND.

MR. TOCHER: THANK YOU, KIM.

IS THERE ANY BOARD COMMENT ON THE MOTION?

CHAIRMAN IMBASCIANI: IS THERE PUBLIC COMMENT? ANY OTHER COMMENTS FROM THE BOARD? I THOUGHT I ASKED. OKAY. I THINK YOU CAN PROCEED.

MR. TOCHER: ALL THOSE IN THE ROOM IN FAVOR SAY AYE. THOSE OPPOSED SAY NAY. ANY ABSTENTIONS? I'LL POLL THE MEMBERS ON THE PHONE.

JUDY CHOU.

DR. CHOU: AYE.

MR. TOCHER: DAVID HIGGINS.

DR. HIGGINS: YES.

MR. TOCHER: RICH LAJARA.

MR. LAJARA: YES.

MR. TOCHER: ADRIANA PADILLA.

DR. PADILLA: YES.
MR. TOCHER: JOE PANETTA.
MR. PANETTA: AYE.
MR. TOCHER: KEVIN XU.
DR. XU: AYE.
MR. TOCHER: AND KEITH YAMAMOTO.
DR. YAMAMOTO: AYE.
MR. TOCHER: THANK YOU. THE MOTION CARRIES UNANIMOUSLY.
CHAIRMAN IMBASCIANI: THANK YOU, SCOTT. AL, WE WILL MAKE SURE YOU ARE IN RECEIPT OF THIS PROCLAMATION. WOULD YOU LIKE TO ADDRESS THE BOARD?
MR. ROWLETT: YES. THANK YOU. I WANTED TO MAKE SURE THAT THE AUDIO WAS ACCEPTABLE. CAN YOU HEAR ME?
VICE CHAIR BONNEVILLE: YES.
MR. ROWLETT: GREETINGS TO THE CHAIR AND VICE CHAIR AND TO CIRM'S PRESIDENT AND CEO, AND TO ALL THE MEMBERS OF THE BOARD. MY HUMBLE APOLOGIES FOR NOT BEING ABLE TO BE THERE. I HAVE A VOLUNTEER COMMITMENT TODAY TO A GROUP OF INDIVIDUALS IN THE COMMUNITY. AND AS YOU MIGHT EXPECT, IT IS THAT KIND OF COMMITMENT THAT CONTINUES TO DRIVE ME EVERY DAY IN THE WORK THAT I CONTINUE TO BE PRIVILEGED TO DO IN THIS VERY UNIQUE TIME IN MY LIFE.

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I ALSO WANT TO ACKNOWLEDGE AND APPRECIATE VERY MUCH THE ACKNOWLEDGEMENT OF MY FAITH, WHICH IS THE GUIDING PRINCIPLES THAT HELP ME IN LIFE EVERY DAY. AS I LIVE, I THANK GOD EVERY DAY FOR THE OPPORTUNITY THAT I HAD TO SERVE AS A MEMBER OF THE CIRM BOARD. AND, YES, I ALSO WANT TO SAY THAT I TOO WAS SO VERY MOVED BY YOUR COMMENTS. I WOULD BE REMISS IF I DIDN'T ACKNOWLEDGE FRED FISHER. THE LATE FRED FISHER HAD A TREMENDOUS IMPACT ON ME, AND I WILL ALWAYS APPRECIATE AND MISS HIS ARTFUL CONTRIBUTIONS.

THAT SAID, TO ALL THE MEMBERS OF THE BOARD, THANK YOU. YOU ARE A LIGHT IN A TIME WHERE THERE IS TURBULENCE AND UNEASINESS FOR SO MANY THAT YOU ARE PRIVILEGED TO REPRESENT. HOWEVER, IN THE WORDS OF LEONDRA CLARK-HARVEY, MY FRIEND AND COLLEAGUE, I REMEMBER WHAT DR. KING SAID. HE SAID, "GIVEN A CHOICE BETWEEN HATE AND LOVE, HE'D CHOOSE TO CHOOSE LOVE VERSUS HATE BECAUSE HATE WAS TOO GREAT A BURDEN TO BEAR."

AS A PATIENT ADVOCATE AT CIRM, I UNDERSTOOD THE IMPORTANCE OF ADVANCING THAT CONCEPT WHEN WORKING WITH UNDERSERVED -- COMMUNITIES THAT I WAS PRIVILEGED TO REPRESENT AND COMMUNITIES THAT DID NOT HAVE HOPE. COMMUNITIES THAT DID NOT OFTEN HAVE

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ACCESS TO THE OUTSTANDING TECHNOLOGIES AND THERAPIES THAT YOU ARE ADVOCATING FOR AND THAT YOU WERE FUNDING. IT WAS THAT HOPE THAT SHIFTED THE TRAJECTORY OF MY UNDERSTANDING OF THE UNIQUE ROLE THAT CIRM PLAYS IN THE STATE OF CALIFORNIA. DON'T BE DISSUADED BY WHAT YOU HEAR, DON'T BE CONCERNED ABOUT THE NEGATIVE RHETORIC, AND DON'T LET IT OVERINFLUENCE WHAT YOU DO. WHAT YOU DO IS STILL IMPORTANT, AND IT IS SO IMPORTANT TO ILLUSTRATE IN CLOSING WITH THIS.

I HAD A UNIQUE PRIVILEGE OF SITTING WITH TWO ADVOCATES ON MONDAY. AND THEY WERE ASKING ME FOR SOME RECOMMENDATIONS, YSABEL, ABOUT ADVOCACY. THEY WANTED HOPE BECAUSE THEY WERE WORKING WITH A FAMILY WHERE THE MOM WAS UNHOUSED AND HER CHILD HAD BEEN DIAGNOSED WITH SICKLE CELL AND SHE DIDN'T KNOW WHAT TO DO. AND I REMEMBER CIRM IN THAT MOMENT AND CONVEYED WHAT YOU EXPECT AN ADVOCATE TO CONVEY, NOT JUST HOPE, BUT RESOURCE THAT WOULD HELP RESOLVE AND REMEDY THE IMMEDIATE DILEMMAS, BUT ALSO TO LET THEM KNOW THAT THERE'S A GROUP OF PEOPLE CALLED CIRM THAT ARE WORKING WITHOUT FAIL ON CURES, CURES THAT THE PEOPLE OF THE STATE OF CALIFORNIA NOT ONLY DESERVE, BUT WANT THAT WILL NOT JUST AFFECT THOSE THAT HAVE ACCESS, BUT THAT AFFECT THOSE POSITIVELY IN

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UNDERSERVED AND POORLY SERVED COMMUNITIES IN THE STATE OF CALIFORNIA.

IT IS MY COMMITMENT TO ALL OF YOU TO CONTINUE TO BE AN ADVOCATE JUST LIKE THE REST OF YOU FOR EVERY PERSON, NOT JUST IN CALIFORNIA, BUT THROUGHOUT THE REST OF OUR COUNTRY AND TALK ABOUT THE GREAT WORK THAT YOU DO AT CIRM. IT WAS A PRIVILEGE TO SERVE WITH YOU. AND I WILL CONTINUE TO BE AN ADVOCATE FOR YOUR GREAT WORK. THANK YOU FOR THE RECOGNITION. THANK YOU FOR THE TIME. AND THANK YOU, CIRM, FOR YOUR COMMITMENT TO CURES FOR THE PEOPLE OF THE STATE OF CALIFORNIA, OUR NATION, AND OUR WORLD. THANK YOU.

(APPLAUSE.)

CHAIRMAN IMBASCIANI: THANK YOU, AL. THANK YOU AGAIN FROM EVERYONE. WELL, FROM THE SUBLIME TO THE MUNDANE. LOOK AT THE NEXT AGENDA ITEM NO. 7, WHICH IS --

MR. TOCHER: MR. CHAIR, IF I COULD JUST, JUST FOR PLANNING PURPOSES, YES. AND FOR THE FOLKS ON THE PHONE, WE'LL BE JUST TAKING A TEN-MINUTE BIO BREAK AFTER CONSIDERATION BEFORE WE RECONVENE AS THE APPLICATION REVIEW SUBCOMMITTEE. SO JUST SO YOU KNOW, COMING UP.

CHAIRMAN IMBASCIANI: THANK YOU, SCOTT.

SO BEFORE THE BREAK, WE'LL CONDUCT ONE MORE PIECE OF BUSINESS, WHICH IS TO PASS THE CONSENT CALENDAR, WHICH INCLUDES THE MINUTES FROM THE DECEMBER 12TH ICOC MEETING. THERE'S A COPY OF THE MINUTES ON THE BACK TABLE. I'VE LOOKED THEM OVER. I CAN'T FIND ANY EMENDATIONS. SO I ASK FOR A MOTION TO ACCEPT, UNLESS ANYONE WANTS TO ABSTRACT SOMETHING FROM THE CONSENT CALENDAR. SEEING NO HANDS, MAY I HAVE A MOTION TO ACCEPT IT?

DR. SOUTHARD: SO MOVED.

CHAIRMAN IMBASCIANI: THANK YOU, MARV. AND SECONDED?

MR. TOCHER: I HEARD YSABEL.

CHAIRMAN IMBASCIANI: YSABEL. I HEARD YSABEL. ANY DISCUSSION?

MR. TOCHER: PUBLIC COMMENT.

CHAIRMAN IMBASCIANI: NO.

MR. TOCHER: ALL THOSE IN THE ROOM IN FAVOR SAY AYE. THOSE OPPOSED SAY NAY. ANY ABSTENTIONS? AND I'LL POLL THE MEMBERS ON THE PHONE.

JUDY CHOU.

DR. CHOU: AYE.

MR. TOCHER: DAVID HIGGINS.

DR. HIGGINS: YES.

MR. TOCHER: RICH LAJARA.
MR. LAJARA: YES.
MR. TOCHER: ADRIANA PADILLA.
DR. PADILLA: YES.
MR. TOCHER: JOE PANETTA.
MR. PANETTA: YES.
MR. TOCHER: KEVIN XU.
DR. XU: YES.
MR. TOCHER: AND KEITH YAMAMOTO.
DR. YAMAMOTO: YES.
MR. TOCHER: GREAT. THANKS VERY MUCH. THE MOTION CARRIES. SO PERHAPS 10:15.
CHAIRMAN IMBASCIANI: WE WILL RECONVENE AT 10:15. THANK YOU.
MR. TOCHER: GREAT.
(A RECESS WAS TAKEN.)
CHAIRMAN IMBASCIANI: WE ARE BACK IN SESSION. THANK YOU ALL. I'M GOING TO DIRECT YOUR ATTENTION TO AGENDA ITEM 12, CONSIDERATION OF APPLICATIONS THAT HAVE BEEN SUBMITTED IN RESPONSE TO OUR DISCOVERY PROGRAM ANNOUNCEMENTS REFERRED TO AS DISC4 OR THE REMIND PROGRAM. GIL SAMBRANO IS GOING TO LEAD THE PRESENTATION.
DR. SAMBRANO: I'M GIL SAMBRANO. THANK YOU VERY MUCH. GOOD MORNING, EVERYONE. I'M GIL

SAMBRANO THE VICE PRESIDENT OF PORTFOLIO DEVELOPMENT AND REVIEW AT CIRM. AND I'M GOING TO PRESENT TO YOU THE RECOMMENDATIONS FROM THE GRANTS WORKING GROUP AS THEY PERTAIN TO THE DISC4 REMIND-L PROGRAM.

WITH EVERY MEETING THAT WE HAVE, THAT INCLUDES THE GRANTS WORKING GROUP AS WELL AS THE BOARD, WE ALWAYS START OFF WITH OUR MISSION, WHICH IS TO ACCELERATE WORLD-CLASS SCIENCE TO DELIVER TRANSFORMATIVE REGENERATIVE MEDICINE TREATMENTS IN AN EQUITABLE MANNER TO A DIVERSE CALIFORNIA AND WORLD. AND THIS IS AN IMPORTANT REMINDER TO OUR REVIEWERS AS WELL AS OURSELVES ABOUT WHY WE ARE HERE AND TO MAKE SURE THAT WE ARE FOCUSED ON THIS GOAL AS WE ENDEAVOR IN THESE REVIEW SESSIONS.

THE REMIND PROGRAM, REMIND ITSELF IS AN ACRONYM WHICH STANDS FOR RESEARCH USING MULTIDISCIPLINARY INNOVATIVE APPROACHES IN NEURODISEASES. ITS PURPOSE IN A GENERAL SENSE IS TO ACCELERATE DISCOVERY OR MECHANISMS THAT UNDERLIE NEUROLOGICAL DISEASES AND DISORDERS THAT LEAD TO IDENTIFICATION, VALIDATION OF NOVEL TARGETS AND BIOMARKERS FOR FUTURE TRANSLATIONAL OR CLINICAL STUDIES.

IN THIS CASE OR FOR THIS INITIAL OPPORTUNITY, WE FOCUSED EFFORTS ON NEUROPSYCHIATRIC

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DISORDERS. AND THE REMIND PROGRAM STRUCTURE ITSELF IS INTENDED TO BE MULTIDISCIPLINARY AND COLLABORATIVE. AS SUCH, THE REMIND-L AWARDS ARE GOING TO SUPPORT EXPANSIVE, CROSS-DISCIPLINARY, INTEGRATED STUDIES BY LARGE, COLLABORATIVE TEAMS THAT WOULD APPLY A RANGE OF TECHNOLOGIES AND APPROACHES TO THE PROBLEM AREA.

FOR THE PROPOSALS THAT ARE COMING TO US THAT WE ARE CONSIDERING FOR FUNDING, THE OUTCOMES OF THOSE PROPOSALS CAN VARY. THEY CAN INCLUDE ALL OR SOME OF THESE ELEMENTS, WHICH ARE TO DISCOVER NOVEL, MECHANISTIC INSIGHTS OR FURTHER OUR CURRENT UNDERSTANDING OF NEUROPSYCHIATRIC DISEASE MECHANISMS, TO ADDRESS MAJOR BOTTLENECKS IN THE STUDY OF THOSE DISORDERS, TO EXPAND UNDERSTANDING OF DISEASE MECHANISMS TO DIVERSE HUMAN POPULATIONS, IDENTIFY AND VALIDATE NEW THERAPEUTIC HYPOTHESES, TARGETS, AND BIOMARKERS.

THE REVIEW OF THESE APPLICATIONS FOLLOWS THE TYPICAL THREE-STEP PROCESS OF ELIGIBILITY THAT IS ASSESSED BY CIRM, MERIT REVIEW BY THE GRANTS WORKING GROUP, AND THE FUNDING DECISIONS THAT'S MADE BY THE APPLICATION REVIEW SUBCOMMITTEE.

THE APPLICATIONS ARE ALL SCORED BY THE GRANTS WORKING GROUP USING A SCALE OF 1, 2, OR 3.

AND THOSE THAT RECEIVE A SCORE OF 2 CAN HAVE THE OPPORTUNITY TO RESUBMIT. I'LL TELL YOU A LITTLE MORE AS WE GO OVER WHAT THE INITIAL OUTCOME OF THE REVIEW WAS THAT HAPPENED SIX MONTHS AGO AND NOW THE RESUBMISSIONS WHICH IS THE SUBJECT OF THIS LATEST REVIEW.

SO BRIEFLY JUST WANT TO SHARE WHAT THE REVIEW CRITERIA THAT THE GRANTS WORKING GROUP USES FOR THIS REMIND COMPETITION. THE REVIEWERS EVALUATE AND ASK WHETHER THE PROJECT HOLDS THE NECESSARY SIGNIFICANCE AND POTENTIAL FOR IMPACT. WHETHER THE PROPOSAL IS INNOVATIVE, HAS A SOUND RATIONALE, IS WELL-PLANNED AND DESIGNED, IS FEASIBLE, AND UPHOLDS THE PRINCIPLES OF DIVERSITY, EQUITY, AND INCLUSION.

THE PANEL COMPOSITION INCLUDES OUR 15 SCIENTIFIC MEMBERS THAT ARE APPOINTED TO THE PANEL BASED ON THEIR EXPERTISE, IN THIS CASE IN NEUROPSYCHIATRIC DISORDERS. THEY CONDUCT THE SCIENTIFIC EVALUATION AND ENTER THE SCIENTIFIC SCORES FOR EVERY APPLICATION. WE ALSO HAVE OUR GRANTS WORKING GROUP BOARD MEMBERS. SO THOSE ARE OUR PATIENT ADVOCATE OR NURSE MEMBERS WHO PARTICIPATE ON THE PANEL AND PROVIDE THE PATIENT PERSPECTIVE ON SIGNIFICANCE AND POTENTIAL IMPACT AS WELL AS PROVIDE OVERSIGHT ON THE PROCESS.

WE ALSO INCLUDE, AS NEEDED, SPECIALISTS, SO SCIENTIFIC EXPERTS IN SPECIALIZED AREAS AS NEEDED TO FILL IN ANY KNOWLEDGE GAPS THAT MAY BE NECESSARY FOR THE REVIEW.

OKAY. SO WE'RE GOING TO START WITH WHAT HAPPENED SIX MONTHS AGO. IN AUGUST WE CAME TO YOU WITH THE RECOMMENDATIONS OF THE GRANTS WORKING GROUP FROM THE INITIAL REVIEW OF THE REMIND-L APPLICATIONS. WE HAD AT THAT TIME 26 SUBMISSIONS THAT WERE REVIEWED. THERE WERE FIVE APPLICATIONS THAT EARNED A SCORE OF 1, NINE APPLICATIONS THAT HAD A SCORE OF 2, NEEDING IMPROVEMENT, AND 12 THAT GOT A SCORE OF 3, NOT WARRANTING FUNDING.

SO FOR THESE THE BOARD, THE APPLICATION REVIEW SUBCOMMITTEE, APPROVED FUNDING OF THOSE FIVE APPLICATIONS WITH A SCORE OF 1 FOR THE TOTAL AMOUNT OF 67.5 MILLION. SO THOSE APPLICATIONS ARE NOW IN THE PROCESS OF BEING LAUNCHED. SOME HAVE LAUNCHED. SO THOSE HAVE AND ARE MOVING FORWARD.

THE 12 THAT RECEIVED A SCORE OF 3 WERE NOT FUNDED. AND THEN THE NINE THAT RECEIVED A SCORE OF 2 WERE INVITED TO REVISE AND RESUBMIT. AND THIS WAS NOTING THAT THE DIFFERENCE BETWEEN THE FUNDS THAT WERE ALLOCATED TO THIS CYCLE OF 88.2 MILLION AND THE 67.5 THAT UTILIZED BY THE FIVE APPLICATIONS LEAVES

YOU WITH ABOUT 20 MILLION OR SO WITH WHICH WE COULD FUND ONE OR TWO ADDITIONAL. SO KNOWING THAT, THOSE NINE APPLICATIONS WERE INVITED TO RESUBMIT.

SO THE STATUS OF THAT IS THAT THOSE APPLICATIONS, EIGHT OUT OF THE NINE INVITED APPLICATIONS ACTUALLY SUBMITTED, AND THOSE WERE REVIEWED BY THE GRANTS WORKING GROUP. AND THE FOCUS OF THE REVIEW, THEN, WAS TO EVALUATE AND SCORE THE RESUBMISSIONS THAT WOULD ADDRESS CONCERNS FROM REVIEWERS FROM THE ORIGINAL REVIEW. AND SO THE APPLICANTS PROVIDED A REVISION DOCUMENT, REDLINE PROPOSAL OF WHAT CHANGES THEY WOULD MAKE. AND THEN WE ALSO PROVIDED THE INITIAL REVIEW SUMMARY DOCUMENT TO THE GRANTS WORKING GROUP FOR REFERENCE OF THE CONCERNS THAT WERE ORIGINALLY RAISED.

ALL RIGHT. SO NOW WE COME TO THE RECOMMENDATIONS FROM THE GRANTS WORKING GROUP PERTAINING TO THE RESUBMISSIONS. SO OF THE EIGHT APPLICATIONS THAT CAME BACK WITH A REVISED APPLICATION, THREE OF THOSE RECEIVED A SCORE OF 1, FOUR RECEIVED A SCORE OF 2, AND ONE RECEIVED A SCORE OF 3. THE TOTAL APPLICANT REQUEST FOR THE THREE THAT RECEIVED A SCORE OF 1 IS 30.8 MILLION. THE FUNDS AVAILABLE ARE 20.675 MILLION. SO IT'S NOT POSSIBLE FOR US TO FUND ALL THREE THAT RECEIVED A

SCORE OF 1.

SO AS SUCH, THE CIRM TEAM LOOKED AT THOSE THREE APPLICATIONS THAT RECEIVED A SCORE OF 1 TO IDENTIFY IF WE HAVE A RECOMMENDATION OF WHICH ONES SHOULD BE FUNDED. SO IN LOOKING AT THIS, WE LOOKED PRIMARILY TO THE GRANTS WORKING GROUP SCORE AND THE RANK ORDER. AND AS YOU CAN SEE, THERE ARE TWO APPLICATIONS THAT HAD A UNANIMOUS SCORE OF 1, IN ONE CASE 15 MEMBERS VOTING IN FAVOR AND 14 IN THE OTHER ONE. THE THIRD APPLICATION, 16400, RECEIVED A SCORE OF 1, HAD A SPLIT VOTE OF EIGHT VERSUS SEVEN.

SO OUR RECOMMENDATION IS TO FUND THE ONES WITH A UNANIMOUS SCORE AND NOT THE ONE THAT HAD THE SPLIT VOTE. BUT BEYOND THE SCORE, THE FIRST TWO APPLICATIONS DO BRING SOMETHING THAT IS DIFFERENT OR UNIQUE COMPARED TO THE FIVE THAT HAVE ALREADY BEEN FUNDED OR ARE BEING LAUNCHED. THE FIRST ONE FOCUSES ON THE NEUROVASCULATURE AND METABOLISM IN AUTISM SPECTRUM DISEASE. AND THE SECOND ONE ON MICRODELETION SYNDROMES. THE THIRD ONE HAS MANY ELEMENTS THAT ARE PART OF PROPOSALS THAT WERE FUNDED IN THE INITIAL ROUND.

IN ADDITION, THE GRANTS WORKING GROUP HAD CONCERNS ABOUT THE LAST AIM, WHICH RELATED TO THE AIM WHERE ALL OF THE DATA THAT WAS COLLECTED IN THE

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PREVIOUS AIMS WOULD COME TOGETHER IN ORDER TO BETTER UNDERSTAND THE OUTCOMES AND PROPOSED POTENTIAL THERAPEUTIC LEADS. AND SO THAT WAS THE WEAKEST PART THAT THEY FOUND AND PREDOMINANTLY THE REASON FOR THAT SPLIT VOTE.

SO GIVEN THOSE CIRCUMSTANCES, THE CIRM TEAM IS RECOMMENDING THE FUNDING OF THE TOP TWO APPLICATIONS: 16337 AND 16345. AND SO THOSE ARE RECOMMENDATIONS, AND I'LL TURN IT BACK IT YOU.

CHAIRMAN IMBASCIANI: THANK YOU, GIL, FOR THAT PRESENTATION AND YOUR EXPLANATIONS. SO I'M LOOKING FOR A MOTION.

VICE CHAIR BONNEVILLE: I'D LIKE TO MAKE A MOTION ACTUALLY TO NOT FUND THOSE THAT ARE NOT RECOMMENDED FOR FUNDING AND START THERE.

MR. TOCHER: TO CLARIFY, THAT'S THE GRANTS WORKING GROUP RECOMMENDATIONS TO NOT FUND.

VICE CHAIR BONNEVILLE: GRANTS WORKING GROUP RECOMMENDATION TO NOT FUND --

MR. TOCHER: CORRECT.

VICE CHAIR BONNEVILLE: -- THOSE THAT WERE NOT RECOMMENDED FOR FUNDING. THANK YOU, SCOTT, FOR THE CLARIFICATION.

DR. SAMBRANO: I APOLOGIZE. I FORGOT TO SHOW THE SLIDE WHICH HAS JUST THE CONFLICTS JUST FOR

FOLKS TO TAKE NOTICE OF IN TERMS OF DISCUSSION.

AND THEN LASTLY, THIS IS THE LIST OF APPLICATIONS JUST FOR YOUR REFERENCE SO THAT YOU CAN SEE THEM MORE CLEARLY AS YOU DISCUSS THEM.

CHAIRMAN IMBASCIANI: I NEED A SECOND TO MARIA'S MOTION.

DR. SOUTHARD: SECOND.

MR. TOCHER: MARV SOUTHARD.

CHAIRMAN IMBASCIANI: THANK YOU. OKAY. THE DISCUSSION IS OPEN FOR BOARD. MARIA, WOULD YOU LIKE TO --

VICE CHAIR BONNEVILLE: I WOULD JUST LIKE TO MAKE THE MOTION AND HAVE THE BOARD VOTE ON THAT MOTION.

MR. TOCHER: RIGHT. I THINK IT WILL HELP FACILITATE DISCUSSION OF THE REMAINING APPLICATIONS BY MEMBERS WHO MIGHT OTHERWISE HAVE A CONFLICT.

CHAIRMAN IMBASCIANI: OKAY. SO RIGHT NOW WE'RE LOOKING FOR COMMENT FROM BOARD MEMBERS. I SEE ANNE-MARIE'S HAND. YES.

DR. DULIEGE: THANK YOU, GIL, AGAIN, FOR, AS USUAL, AN EXCELLENT PRESENTATION AND VERY CLEAR DATA THAT WE CAN HAVE ACCESS TO. SO SIMPLY IN REGARDS TO THE LAST APPLICATION, 16400, I APPRECIATE THE RECOMMENDATION COMING FROM THE CIRM TEAM. IS IT

BOTH BECAUSE WE DON'T HAVE THE MONEY TO FUND THE THREE AND THE FACT THAT, COMPARED TO THE OTHER APPLICATIONS, THIS ONE HAD SOME SUBOPTIMAL ASPECTS TO IT? IS THE COMBINATION OF THESE TWO FACTORS THAT JUSTIFY THE CIRM --

CHAIRMAN IMBASCIANI: ANNE-MARIE, SORRY. THE MOTION IS NOT TO FUND --

DR. DULIEGE: OKAY. I JUMPED A LITTLE TOO QUICKLY.

CHAIRMAN IMBASCIANI: YOU'RE UP IN THE GROUP.

DR. DULIEGE: SORRY FOR THAT. I WILL COM UP. I THINK YOU GOT MY QUESTIONS.

CHAIRMAN IMBASCIANI: THANK YOU, ANNE-MARIE.

MR. TOCHER: VITO, SORRY. POINT OF ORDER. I THINK THE SHADING IS VERY DIFFICULT TO SEE FOR MEMBERS IN THE ROOM, THAT THE FIRST APPLICATION BELOW THE LAST GREEN HIGHLIGHTED ONE IS IN A DIFFERENT COLOR. THAT IS NOT THE SUBJECT OF THIS MOTION. THAT'S 16400 WHICH LOOKS LIKE IT'S COLORED IN SLIGHTLY OFF-WHITE. SO THIS MOTION IS WITH RESPECT TO --

CHAIRMAN IMBASCIANI: THE LOWER FIVE.

MR. TOCHER: -- 16360 AND DESCENDING.

DR. DULIEGE: OKAY. THANK YOU. I'LL WAIT.

CHAIRMAN IMBASCIANI: THANK YOU. CONVERSATION FROM BOARD MEMBERS ON MARIAS'S MOTION TO NOT FUND THE BOTTOM FIVE? NOT HEARING THAT, ANY MEMBER OF THE PUBLIC LIKE TO COMMENT ON THIS MOTION?

MS. MANDAC: WE DO HAVE HANDS RAISED. I JUST WANT TO CONFIRM ALL OF THE INDIVIDUALS WITH HANDS RAISED, ARE YOU COMMENTING ON THE LAST FIVE APPLICATIONS ON THIS LIST DISPLAYED ON THE SCREEN? BECAUSE I RECOGNIZE TWO OF YOU AS APPLICATIONS ABOVE THOSE FIVE. SO IF YOU'RE ONLY COMMENTING ON DISC 16360 AND BELOW, PLEASE KEEP YOUR HAND RAISED. IF YOU ARE NOT COMMENTING ON ONE OF THOSE, PLEASE LOWER YOUR HANDS. OKAY.

SO WE HAVE ONE PUBLIC COMMENT. IT IS A PHONE NUMBER: 415-640-8961. WE WILL HAVE A TIMER. YOU WILL HAVE THREE MINUTES. WE WILL MUTE YOU AS SOON AS THE THREE MINUTES IS OVER. SO PLEASE PAY ATTENTION TO THE CLOCK ON YOUR SCREEN. YOUR TIME STARTS NOW. PHONE NUMBER 415-640-8961, YOU WILL NEED TO UNMUTE IF LIKE TO PARTICIPATE IN PUBLIC COMMENT. ALL RIGHT.

CHAIRMAN IMBASCIANI: NOTHING IS COMING THROUGH?

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MS. MANDAC: NO.

CHAIRMAN IMBASCIANI: ALL RIGHT. OKAY. NO MEMBER OF THE PUBLIC IS SPEAKING ON THIS. WE CAN PROCEED TO A VOTE. SCOTT, IF YOU WILL JUST STATE THE MOTION AGAIN FOR CLARITY.

MR. TOCHER: SURE. JUST MAKING SURE WE'RE CLEAR FOR CONFLICTS. THE MOTION IS TO NOT FUND THE APPLICATIONS THAT THE GRANTS WORKING GROUP RECOMMEND TO FUND WHICH ARE THE BOTTOM FIVE OF THE LIST THAT YOU SEE; I.E., NOT INCLUDING 16337, 16345, OR 16400.

MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: YES.

MR. TOCHER: JUDY CHOU.

DR. CHOU: YES.

MR. TOCHER: LEONDRA CLARK-HARVEY.

DR. CLARK-HARVEY: YES.

MR. TOCHER: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MR. TOCHER: MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: YES.

MR. TOCHER: DAVID HIGGINS.

DR. HIGGINS: YES.

MR. TOCHER: VITO IMBASCIANI.

CHAIRMAN IMBASCIANI: YES.

MR. TOCHER: SORRY. I WANT TO CHECK.

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DR. CLARK-HARVEY: I WAS A BIT CONFUSED WHEN I WAS CALLED I'M ON THE CONFLICT LIST.

MR. TOCHER: SORRY. WE HAD AN OLD LIST. I'M SORRY. WE'LL CORRECT THAT ON THE RECORD.

RICH LAJARA.

MR. LAJARA: YES.

MR. TOCHER: ADRIANA PADILLA.

DR. PADILLA: YES.

MR. TOCHER: JOE PANETTA.

MR. PANETTA: YES.

MR. TOCHER: MARV SOUTHARD.

DR. SOUTHARD: YES.

MR. TOCHER: YAEL WYTE.

MS. WYTE: YES.

MR. TOCHER: AND KEVIN XU.

DR. XU: YES.

MR. TOCHER. GREAT. THANK YOU. THE MOTION CARRIES.

CHAIRMAN IMBASCIANI: THANK YOU. FLOOR IS NOW OPEN TO A MOTION FOR THE REMAINING APPLICATIONS. I WOULD SUGGEST THE PERSON MAKING THE MOTION CONSIDER THE BUDGETARY CONSTRAINTS.

VICE CHAIR BONNEVILLE: PAT HAS A QUESTION.

CHAIRMAN IMBASCIANI: PAT.

DR. LEVITT: SORRY. I CAN MAKE A MOTION.

CHAIRMAN IMBASCIANI: IF YOU WANT TO MAKE A COMMENT FIRST.

DR. LEVITT: THANK YOU.

MR. TOCHER: NO.

VICE CHAIR BONNEVILLE: YOU CAN'T MAKE A MOTION, BUT YOU CAN COMMENT.

DR. LEVITT: I CAN COMMENT ABOUT --

CHAIRMAN IMBASCIANI: YES.

DR. LEVITT: THANK YOU.

DR. DULIEGE: I CAN MAKE THE MOTION.

DR. LEVITT: I'M READY TO GO. SO CAN YOU HEAR ME?

VICE CHAIR BONNEVILLE: YEAH.

DR. LEVITT: OKAY. SO THIS IS RELATED TO DISC 16400 WHICH WAS RECOMMENDED FOR FUNDING. IT DID HAVE A SPLIT VOTE IN TERMS OF TIER. I'LL FIRST POINT OUT WHAT THE STUDY IS ABOUT. THE STUDY INCLUDES ADDRESSING THE ETIOLOGY FROM THE DEVELOPMENTAL PERSPECTIVE OF BOTH AUTISM AND SCHIZOPHRENIA.

AS FAR AS I KNOW, AND I DON'T KNOW THE COMPLETE PROFILE OF GRANTS, BUT I WAS ON THE NEUROSCIENCE TASK FORCE, CHAIRED IT FOR SEVERAL MEETINGS. WE HAVE ALMOST NOTHING IN PSYCHOSIS,

ALMOST ZERO, AND IT MAY BE ZERO. IN ADDITION TO WHICH IT IS A STUDY THAT IS FOCUSED ON AFRICAN-AMERICAN PATIENT SAMPLES, WHICH IS EXTRAORDINARILY UNUSUAL IN THIS FIELD. ALMOST ALL THE INFORMATION WE HAVE IN TRANSCRIPTOMICS AND GENETICS IS NORTHERN EUROPEAN WHITE. THERE ARE SEVERAL STUDIES ONGOING NOW THAT INCLUDE AFRICAN-AMERICANS AND LATINAE POPULATIONS WITH COMPLEX ANCESTRIES.

SO THIS IS ADDRESSING, FROM MY PERSPECTIVE, A MAJOR BOTTLENECK. AND THAT'S USED IN THE REVIEWS, IF YOU READ THE REVIEWS, IN TERMS OF PSYCHOSIS ETIOLOGY AND IN TERMS OF THE POPULATION THAT IS INCLUDED PROBABLY FOR THE FIRST TIME AT CIRM, CERTAINLY AT THE FEDERAL LEVEL.

IF YOU GO DOWN AND LOOK AT DOES THE PROJECT HOLD THE NECESSARY SIGNIFICANCE AND POTENTIAL IMPACT, THE VOTE WAS 13 TO 1. IS THE PROPOSAL INNOVATIVE, 13 TO 1. IS THE RATIONALE SOUND, 14 TO 0. IS THE PROJECT WELL-PLANNED AND DESIGNED, 9 TO 5. THAT'S FIRST TIER, SECOND TIER. AND THE FIVE REALLY FOCUSED ON, AND I DON'T HAVE THE GRANT, I DIDN'T READ THE GRANT, BUT REALLY FOCUSED ON THE ANALYTICAL PART OF ARTIFICIAL INTELLIGENCE OF PUTTING ALL THIS DATA TOGETHER. THERE WERE LOTS OF

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COMMENTS ABOUT THE HIGH THROUGHPUT TECHNOLOGY THAT IS PROPOSED HERE. AND, AGAIN, I DON'T KNOW THE DETAILS. IS THE PROJECT FEASIBLE, 13 TO 1. DOES THE PROJECT UPHOLD THE PRINCIPLES OF DIVERSITY, EQUITY, AND INCLUSION, 14/0.

SO WHEN I READ THOSE AND A FEW RED FLAGS THAT I'LL MENTION, SO FOR THOSE OF YOU WHO DON'T KNOW, I'M A DEVELOPMENTAL NEUROSCIENTIST. MY LAB WAS THE FIRST TO DO TRANSCRIPTOME ANALYSIS FOR SCHIZOPHRENIA IN 2000. SO I KNOW A BIT ABOUT THIS FIELD. I'M A GRADUATE OF UCSD AS WELL. I WANTED TO POINT THAT OUT.

THERE'S SEVERAL THINGS. ONE IS THAT THERE ARE COMMENTS IN HERE ABOUT HOW THE MODEL MAY BE RELEVANT FOR AUTISM BECAUSE IT'S A NEURODEVELOPMENTAL DISORDER, MAYBE NOT FOR SCHIZOPHRENIA, WHICH, OF COURSE, IS A NEURODEVELOPMENTAL DISORDER. AND IF YOU LOOK AT THE STUDIES THAT HAVE BEEN DONE OVER THE LAST DECADE, WHOLE GENOME SEQUENCING ANALYSES, POLYGENIC RISK SCORES, ET CETERA, THERE ARE LARGE SETS OF GENES THAT HAVE BEEN IDENTIFIED THAT ARE RISK FOR SCHIZOPHRENIA. AND EXPRESSION, THE ENRICHMENT OF EXPRESSION IS SECOND TRIMESTER PRENATAL.

AND I'VE WRITTEN ABOUT THIS AND OTHERS

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HAVE AS WELL. THIS IS A PRENATAL COMPONENT. SO THIS MODEL IS -- THERE'S NOTHING IN THE LITERATURE THAT SAYS AN IPSC MODEL OR AN ORGANOID MODEL IS INAPPROPRIATE FOR SCHIZOPHRENIA BASED ON THE BIOLOGY.

IN TERMS OF THE CRITICISMS OF THE ANALYTICS, I'M SENSITIVE TO THIS BECAUSE WE'VE BEEN DOING ARTIFICIAL INTELLIGENCE AND MACHINE LEARNING ON DATA ANALYSIS SINCE 2015. THE FIELD IS OLD. AND I CAN TELL YOU THAT, JUST FROM MY EXPERIENCE AND FROM DEALING WITH THIS IN A VERY BROAD WAY, DEEP WAY, ACROSS BIOMEDICAL DISCIPLINES, THE AI AND MACHINE LEARNING APPROACHES THAT WERE USED SIX MONTHS AGO ARE DIFFERENT THAN THEY ARE NOW. THEY'RE DIFFERENT NOW THAN THEY WERE EVEN SIX MONTHS AGO.

AND SO THERE'S SOME COMMENTS IN HERE ABOUT ONE OF THE REVIEWERS PREDICTING THAT THIS IS GOING TO FAIL BECAUSE OF WHATEVER THE APPROACH WAS. AND I CAN'T COMMENT ON THE SPECIFICS. I HAVEN'T SEEN THEM. BUT I THINK THAT IS MAKING A CASE THAT AN INDIVIDUAL CAN ACTUALLY PREDICT WHAT AI MODELS -- AI MODELS BY DEFINITION ARE MEANT TO BE FLEXIBLE, ADAPTABLE, AND DEPEND EXCLUSIVELY ON DATA TO TRAIN THE AI MODEL IN THE FIRST PLACE. AND SO THAT CAN'T BE DONE UNTIL YOU HAVE THE DATA, AND THE ANALYTICS

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THAT WILL OCCUR SIX MONTHS FROM NOW OR A YEAR FROM NOW ARE GOING TO BE DIFFERENT, THERE'S NO DOUBT ABOUT IT, THAN WHAT THIS GROUP OR ANY OTHER GROUP PROPOSES TO DO IN THEIR STUDIES.

SO THAT'S THE CHALLENGE IN TERMS OF REVIEWING THE ANALYTICAL PART BECAUSE WE KNOW IT'S A MOVING OBJECT. IT'S REALLY HARD. SO I CAN SPEAK TO THE SCIENCE. OF COURSE, I CAN SPEAK TO THE FACT THAT WE HAVE PART OF A PROPOSITION TO DEAL WITH. I'M CONCERNED ABOUT WE HAVE ALMOST NOTHING IN PSYCHOSIS STILL AND WE'VE HAD SEVERAL YEARS OF THE NEUROSCIENCE TASK FORCE DEALING WITH THIS. AND I DON'T KNOW WHAT THE REMEDIES ARE FOR THIS.

BUT WHEN I LOOK AT THE REVIEWS OF THIS AND CONSIDERING THE POPULATION AND THE FOCUS ON PSYCHOSIS, I THINK THERE NEEDS TO BE SOME CONVERSATION ABOUT HOW TO ADDRESS THIS.

IF IT'S THE STANDARD WAY IN WHICH A REVISION IS HANDLED, YOU'RE TALKING ABOUT A DELAY OF A YEAR OR MORE IN TERMS OF HOW LONG IT WOULD TAKE TO COME BACK. I DON'T KNOW IF THAT'S THE CASE OR NOT HERE, GIL. BUT I'LL STOP THERE AND HAVE OTHERS IF THEY WANT TO COMMENT.

VICE CHAIR BONNEVILLE: SO I THINK THE PROBLEM IS THAT IT'S A BUDGETARY CONSTRAINT, AND

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THIS BUDGET IS LIMITED. AND SO I THINK OTHERWISE, IF THERE HAD BEEN MORE IN THE BUDGET, IT WOULD HAVE GONE THROUGH. SO I UNDERSTAND YOUR POINT, BUT WE'RE CONSTRAINED BY A SPECIFIC BUDGET AMOUNT.

I DON'T KNOW HOW TO WEIGH IN ON IT OTHER THAN THE TEAM WENT WITH THE TWO THAT WERE RECOMMENDED. SO THAT'S WHERE THE PROBLEM IS. IT'S NOT THAT THEY DIDN'T FIND THIS MERITORIOUS. IT'S THAT THERE WAS NOT ENOUGH OF A BUDGET.

DR. LEVITT: SO CAN I REPLY? CAN I ASK A QUESTION?

VICE CHAIR BONNEVILLE: YEAH. YOU CAN DO WHATEVER YOU WANT, PAT.

DR. LEVITT: NO, I CAN'T.

VICE CHAIR BONNEVILLE: ALMOST EVERYTHING.

DR. LEVITT: NO, I CAN'T. NOT IN MY PRIVATE LIFE OR IN MY PROFESSIONAL LIFE, ESPECIALLY MY PRIVATE LIFE.

I DON'T KNOW HOW THE BUDGETS ARE MANAGED. I JUST KNOW AT A FEDERAL LEVEL BUDGET REQUESTS ARE MANAGED IN A VERY PROACTIVE WAY BY PROGRAM DIRECTORS. THESE BUDGETS ARE MODIFIED ALL THE TIME, ALL THE TIME. I DON'T KNOW IF CIRM DOES THAT OR WHETHER THE REQUEST THAT COMES IN IS THE REQUEST THAT IS UTILIZED TO THEN DETERMINE WHAT THE BUDGET

IS GOING TO BE OR WHETHER THERE'S A WAY OF -- THERE'S GOING TO BE MORE ON REMIND ACTIVITY GOING ON; IS THAT RIGHT, GIL?

DR. SAMBRANO: YES, THAT'S RIGHT.

DR. LEVITT: YEAH. SO THERE'S GOING TO BE MORE GRANTS COMING THROUGH, RIGHT, IN RESPONSE TO THE 1.5 BILLION, 1.4 BILLION?

DR. SAMBRANO: RIGHT. SO LET ME JUST CLARIFY IN TERMS OF THE BUDGET. THE WAY IT WORKS IS WE HAVE AN ANNUAL BUDGET THAT THE BOARD APPROVES. AND SO WE ALLOCATED SPECIFICALLY A GIVEN AMOUNT FOR THIS INITIAL ROUND. SO WE STAY WITHIN THAT ALLOCATED BUDGET THAT WAS APPROVED BY THE BOARD, AND THAT'S BEEN OUR PRACTICE.

SO TO THE EXTENT THAT THERE WOULD BE FLEXIBILITY IS IF THERE ARE FUNDS THAT REMAIN. AND AS YOU'VE OBSERVED IN MANY CASES NOW, WE HAVE THE ISSUE THAT THERE ARE MORE GRANT APPLICATIONS THAT RECEIVE A FUNDABLE THAN WE CAN ACTUALLY COVER. SO IT DOES MAKE THE DECISION-MAKING VERY DIFFICULT AND CHALLENGING FOR EVERYONE. SO THAT UNFORTUNATELY IS THE SITUATION HERE.

DR. LEVITT: CAN I ASK IF -- SO IF THE TEAM LOOKED AT THIS AND LOOKED AT WHAT WE HAVE COVERAGE IN TERMS OF AUTISM SPECTRUM DISORDER, WHICH

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THE OTHER TWO ARE ABOUT, THE MICRODELETION AND THE VASCULAR ONES -- AND I'M NOT GOING TO COMMENT ABOUT THOSE. I READ ALL THROUGH THOSE IN GREAT DETAIL -- WHAT WAS THE TEAM'S CONSIDERATION IN TERMS OF THE FOCUS THAT ONE OF THE TWO AREAS OF FOCUS FOR THIS OTHER GRANT IS PSYCHOSIS AND THAT IT'S AN AFRICAN-AMERICAN CLINICAL POPULATION THAT'S GOING TO BE STUDIED? THAT'S CRITICAL BECAUSE WE KNOW FROM PHARMACOGENOMICS THAT BOTH INCLUSION OF DIVERSITY BOTH IN TERMS OF SEX AND IN TERMS OF THE ANCESTRY MATTERS A LOT IN TERMS OF TREATMENTS THAT ARE GOING TO COME DOWN THE PIKE.

DR. SAMBRANO: SO THAT'S A FAIR QUESTION. AND WE DID CONSIDER THAT IN TERMS OF WHAT ELSE WE FUNDED. SO I BRIEFLY MENTIONED THAT THERE WERE FIVE APPLICATIONS THAT WERE FUNDED INITIALLY BEFORE THESE HAD THEIR RESUBMISSIONS. AND SO LOOKING ACROSS THOSE, THERE ARE SEVERAL THAT ARE DOING HIGH THROUGHPUT PHENOTYPING OF RISK GENES, MOST OF THEM. SO THERE'S FOUR PROJECTS THAT ARE FOCUSED ON AUTISM, TWO ON SCHIZOPHRENIA. AND SO A LOT OF THE GENERAL ELEMENTS ARE COVERED EVEN THOUGH IT MAY NOT BE EXACTLY THE SAME.

SOME ARE GENERATING IPSC LINES FROM DIVERSE GROUPS, NOT IN THE SAME WAY AS PROPOSED HERE

BECAUSE I THINK THE WAY IT'S PROPOSED HERE IS RATHER UNIQUE, BUT IN A GENERAL SENSE, THERE ARE OTHERS THAT ARE ATTEMPTING TO DO SOME MORE THINGS.

CHAIRMAN IMBASCIANI: I HAVE TWO SPEAKERS. JOE PANETTA IS GOING TO BE FIRST FOLLOWED BY DR. CARETHERS. JOE, ARE YOU ON THE LINE?

MR. PANETTA: THANKS, VITO. I'VE JUST GOT A BASIC QUESTION SO THAT WE UNDERSTAND THIS. I KNOW THESE ARE RESUBMISSIONS, BUT, GIL, IS THIS THE TOTAL AMOUNT AVAILABLE FOR THE YEAR SO THAT THIS CANNOT BE CONSIDERED AGAIN BECAUSE THERE ARE ABSOLUTELY NO ADDITIONAL FUNDS AVAILABLE?

DR. SAMBRANO: WELL, CORRECT. FOR THIS CYCLE, THIS COMPETITION, THAT IS THE MAXIMUM AMOUNT THAT WE CAN EXPEND ON THIS. SO UNTIL THE NEXT ITERATION OF REMIND COMES BACK, AND I BELIEVE, AND ROSA CAN MAYBE SPEAK TO THIS, THE EXPECTATION IS THAT THESE PROJECTS WOULD LIKELY BE ELIGIBLE FOR COMING BACK WHEN THAT REOPENS.

MR. PANETTA: OKAY. THANKS. SO THERE IS THE OPPORTUNITY FOR THIS TO COME BACK?

DR. SAMBRANO: YES.

MR. PANETTA: THANK YOU.

CHAIRMAN IMBASCIANI: DR. CARETHERS.

DR. CARETHERS: I HAVE A VERY SIMILAR

QUESTION. MY QUESTION SIMPLY WAS I NOTICE IT'S A SECOND SUBMISSION. CAN IT COME BACK WHEN FUNDS ARE AVAILABLE? SO EXACTLY THE SAME QUESTION. MOST -- FOR INSTANCE, AND THIS IS MY FIRST TIME HERE, BUT MOST NIH GRANTS, EITHER YOU GET A FIRST CHANCE, A SECOND CHANCE; BUT THEN, IF YOU REPACKAGE IT, IT CAN COME IN AS A NEW GRANT. SO I DIDN'T KNOW WHAT THE OPTIONS WERE FOR CERTAIN.

DR. LEVITT: WHAT'S THE TIMELINE?

DR. CANET-AVILES: SO JUST TO CLARIFY, AS THE BOARD IS AWARE, WE HAVE -- AS PART OF THE STRATEGIC ALLOCATION FRAMEWORK, WE ARE COMING IN THE MARCH BOARD THAT WILL BE IN SACRAMENTO WITH NEW CONCEPTS. AND PART OF THAT, WE ARE REVAMPING THIS FORM, WHICH IS THE REMIND, AND THERE WILL BE MORE OPPORTUNITIES COMING IN THE LATE SPRING FOR DISC GRANTS. SO IF THE BOARD APPROVES AND THE BUDGET THAT GOES WITH IT, THEN THERE WILL BE MORE OPPORTUNITIES.

CHAIRMAN IMBASCIANI: THANK YOU, ROSA. J.T.

DR. THOMAS: SO THIS COMMENT IS MAINLY FOR THE NEWER BOARD MEMBERS JUST SO YOU'RE AWARE THAT FROM TIME TO TIME THIS ISSUE COMES UP WHERE WE DON'T HAVE ENOUGH FUNDING TO COVER PROJECTS RECOMMENDED

FOR FUNDING. AND SOMEBODY, AT LEAST IN THE BACK OF THEIR MIND, SAID WHY DON'T WE JUST INCREASE THE BUDGET? AND I JUST WANTED TO SAY THAT IT HAS ALWAYS BEEN CIRM'S PRACTICE, AND HERE I'M CHANNELING FRED FISHER AND STEVE JUELSGAARD, TO STICK TO BUDGETARY DISCIPLINE AND NOT GET INVOLVED IN TRYING ON THE FLY TO INCREASE BUDGETS TO ACCOMMODATE SITUATIONS LIKE THIS.

SO I STRONGLY RECOMMEND THAT WE ADHERE TO THE BUDGET THAT THE BOARD HAS PASSED AND DEAL WITH THIS WHEN IT COMES BACK FOR RESUBMISSION.

DR. LEVITT: I WOULD JUST -- CAN I RESPOND TO THAT?

CHAIRMAN IMBASCIANI: PAT, YOU MAY SPEAK AGAIN, YES.

DR. LEVITT: OKAY. SO MY COMMENTS ARE FRAMED IN THE CONTEXT OF WHAT WE'RE RESPONSIBLE FOR IN TERMS OF PROPOSITION 14 AND THE EXTRAORDINARY AMOUNT OF WORK THAT WENT INTO THE NEUROSCIENCE TASK FORCE, AND THE DISCIPLINE THAT WE HAVE TO HAVE IN TERMS OF TRYING TO ACHIEVE THOSE GOALS, WHICH WE KNOW FROM THE TASK FORCE, ARE GOING TO BE EXTRAORDINARILY DIFFICULT UNDER THE BEST OF CIRCUMSTANCES. SO WHILE I AM IN MY ENTIRE CAREER TRYING TO BE AS FISCALLY RESPONSIBLE AS POSSIBLE,

AND I EMBRACE THAT. J.T., YOU KNOW THAT. I'M JUST QUITE WORRIED ABOUT MEETING THOSE GOALS AND THE RECOMMENDATIONS THAT CAME OUT OF THE TASK FORCE.

AND SO -- AND I WAS MOST -- AND THAT IS MY FRAME FOR, THEN, LOOKING AT THIS AND SAYING THERE ARE SOME EXTRAORDINARILY UNIQUE COMPONENTS TO THIS THAT ACTUALLY MEET SO MANY OF THE GOALS THAT GIL PUT UP ON THE SCREEN IN TERMS OF REMIND AND IN TERMS OF ADDRESSING THAT PART OF THE PROPOSITION.

SO I'LL STOP THERE, BUT I JUST -- IT IS THE CASE THAT AT THE NIH OR THE NSF OR FEDERAL AGENCIES, THE TURNAROUND TIME IS ABOUT ANYWHERE BETWEEN THREE AND SIX MONTHS. YOU GET YOUR REVIEWS BACK, YOU ADDRESS THEM ON A SECOND PAGE, AND THEN YOU RESUBMIT. RIGHT? AND IF IT'S GOING TO BE THE CASE THAT THIS IS GOING TO COME BACK IN THE SPRING, OKAY. BUT I JUST NEEDED FOR FOLKS TO RECOGNIZE WHERE WE ARE IN TERMS OF THE NEURO INITIATIVE AND PARTICULARLY IN THE AREA OF IN NEUROPSYCHIATRY WHERE WE STILL HAVE SUCH A LIMITED PORTFOLIO.

CHAIRMAN IMBASCIANI: THANK YOU, PAT. THAT WAS REALLY A GREAT CONTRIBUTION TO THIS CONVERSATION.

LEADING UP TO THE MOTION, TO MAKE A PECUNIARY PRELUDE, THE NUMBER OF DOLLARS INVOLVED IN

THE TOTAL APPLICANT REQUEST, ONCE AGAIN FROM GIL'S SLIDE, IS $30.8 MILLION. THE FUNDS AVAILABLE, HOWEVER, ARE ONLY $20.6 MILLION. THERE ARE THREE APPLICATIONS WHICH IN ORDER ARE ASKING FOR 10.3, 6.25, AND 14.27. THAT'S THE TOTAL OF 30 MILLION. KNOWING THAT, MAY I HAVE A RESOLUTION?

VICE CHAIR BONNEVILLE: A MOTION.

CHAIRMAN IMBASCIANI: A MOTION. THANK YOU.

DR. DULIEGE: I MOVE.

VICE CHAIR BONNEVILLE: WHAT'S THE MOTION?

CHAIRMAN IMBASCIANI: WHAT'S THE MOTION, ANNE-MARIE.

DR. DULIEGE: WHAT'S THE MOTION? THAT WE APPROVE THE OR REQUEST NO. 1 AND 2 IN THIS LIST, THE FIRST TWO IN GREEN, AND WE DO NOT APPROVE THE THIRD ONE BECAUSE OF LACK OF FUNDING OR DOES NOT MATTER WHY, BUT JUST TO CLARIFY.

CHAIRMAN IMBASCIANI: THAT'S CORRECT ON ALL POINTS. AND THAT'S A VERY CLEAR MOTION. THANK YOU. SECOND?

MR. FISCHER-COLBRIE: SECOND.

CHAIRMAN IMBASCIANI: I HAVE A SECOND FROM MARK FISCHER-COLBRIE. DISCUSSION IS NOW OPEN TO BOARD MEMBERS ON THIS MOTION.

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MR. PANETTA: MR. CHAIR, CAN YOU PLEASE REPEAT THE MOTION? I WASN'T ABLE TO HEAR IT. THANK YOU.

CHAIRMAN IMBASCIANI: YES. JOE, IT'S TO -- ANNE-MARIE DULIEGE MOVED THAT WE MOVE TO FUND THE TOP TWO APPLICATIONS, 16337 AND 16345, AND NOT FUND 16400.

MR. PANETTA: THANK YOU.

CHAIRMAN IMBASCIANI: AND IT WAS SECONDED. DID ALSO YOU WANT TO MAKE A COMMENT PERHAPS, JOE?

MR. PANETTA: YEAH. I WOULD BRIEFLY JUST COMMENT, REALLY FOLLOWING UP ON J.T.'S COMMENT, HAVING BEEN ON THIS BOARD FOR A WHILE, THAT IT'S BEEN OUR PRACTICE TO FOLLOW FISCAL CONSTRAINTS, STAY WITHIN OUR BUDGET. AND I WOULD SUPPORT THE MOTION AND THAT WE CONTINUE TO ADHERE TO THAT PRACTICE.

CHAIRMAN IMBASCIANI: THANK YOU, JOE. ARE WE ARGUING OVER WHO'S NEXT? IS SOMEONE WANTING TO SPEAK? OKAY.

VICE CHAIR BONNEVILLE: YES, YOU CAN.

DR. CLARK-HARVEY: I'M SO TENTATIVE TODAY. I APPRECIATE AND RESPECT OUR COLLEAGUES THAT HAVE WEIGHED IN. I KNOW THAT'S NOT AN EASY TASK. THERE'S A LOT OF THINGS TO WEIGH AND THERE'S LIMITED FUNDS. SO I DEFINITELY APPRECIATE THAT. AND I WANT

TO LIFT UP PAT'S COMMENTS AS WELL. I THINK IT IS REALLY IMPORTANT, REGARDLESS OF THE BUDGET AND ALL OF THE CONSTRAINTS THAT WE MUST WORK WITHIN, IT IS STILL OUR DUTY AS BOARD MEMBERS TO LOOK AT THINGS CRITICALLY EVEN THOUGH THEY'VE ALREADY HAD A REVIEW. AND I THINK IT'S IMPORTANT IN THE COMMENTS THAT WERE MADE AS WE MOVE FORWARD TO THINK ABOUT THESE PARTICULAR COMMUNITIES AND OUR OPPORTUNITY, MAYBE NOT NOW, BUT LATER TO ADDRESS FUNDING FOR PARTICULAR PROJECTS THAT REALLY FOCUS IN HEALTH EQUITY AND DIVERSITY. SO JUST WANTED TO ADD THOSE COMMENTS.

CHAIRMAN IMBASCIANI: THANK YOU, LEONDRA. YES, CAROLYN.

DR. MELTZER: I UNDERSTAND THE BUDGET CONSTRAINTS. AS THE CO-CHAIR OF THE NEURO TASK FORCE WITH PAT, I JUST WANTED TO LODGE THAT I AGREE. THIS IS A SIGNIFICANTLY IMPORTANT AREA TO LOOK AT. I UNDERSTAND WE CAN'T RIGHT NOW, BUT IT'S IMPORTANT SCIENCE, IMPORTANT GROUP AND WOULD ADD TO THE KNOWLEDGE IN THIS SPACE. THANKS.

CHAIRMAN IMBASCIANI: THANK YOU. MARVIN.

DR. SOUTHARD: I WAS JUST WONDERING IF IT'S POSSIBLE WITHIN THE BUDGET THAT'S AVAILABLE TO APPROACH THEM TO BEGIN SOME PIECES OF THIS PROJECT WITH THE BUDGET THAT WE HAVE AVAILABLE AND THEN

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CONTINUE THE REST AT A FURTHER TIME? IS THAT A POSSIBILITY?

CHAIRMAN IMBASCIANI: I DON'T THINK THAT THE BOARD IS CONSTITUTED IN A WAY THAT WE CAN HAVE THAT DISCUSSION HERE GIVEN OUR BUDGETARY AND OUR PROGRAMMATIC -- DO YOU WANT TO ADD SOMETHING TO THAT? THE CONSTRAINTS --

VICE CHAIR BONNEVILLE: MARV, IT'S MORE ALONG THE LINES OF -- AND WE'VE ENCOUNTERED THIS BEFORE -- GIVING THEM A SPECIFIC AMOUNT OF MONEY. IT'S $2 MILLION THAT'S AVAILABLE. THEY WOULD ACTUALLY HAVE TO APPLY TO TALK ABOUT WHAT THE $2 MILLION, THE GWG WOULD HAVE TO REVIEW THAT IN ORDER TO UNDERSTAND WHETHER OR NOT IT WAS FEASIBLE GIVEN THAT AMOUNT OF MONEY. SO IT CHANGES WHAT THE APPLICATION IS.

IN THE PAST THAT'S JUST NOT THE APPROACH THAT WE'VE TAKEN BECAUSE IT WOULD SEEMINGLY BE A NEW APPLICATION THAT THE GWG WOULD HAVE TO REVIEW FOR THAT LIMITED AMOUNT OF MONEY.

CHAIRMAN IMBASCIANI: IS THERE ANY MEMBER OF THE PUBLIC THAT WANTS TO SPEAK TO THIS MOTION?

MR. TOCHER: THERE ARE TWO.

MS. MANDAC: WE HAVE TWO HANDS RAISED FOR THIS ONE.

CHAIRMAN IMBASCIANI: CLAUDETTE, IF YOU CAN MANAGE THAT.

MS. MANDAC: SO WE HAVE TWO HANDS RAISED. WE'LL GO WITH DAN GESHWIND FIRST AND THEN DAVID HAUSSLER. YOU WILL BOTH HAVE THREE MINUTES EACH. THERE IS A TIMER. AND THEY'RE BOTH SPEAKING ABOUT DISC4-16400. IT'S THE THIRD APPLICATION ON THE SCREEN ON THE TOP.

DAN, YOUR TIME STARTS NOW.

DR. GESCHWIND: CAN YOU HEAR ME?

MS. MANDAC: YES.

DR. GESCHWIND: GREAT. SO I'M TALKING ABOUT OUR APPLICATION THAT'S JUST BEEN DISCUSSED, THE DISC REMIND 16400. AS YOU SAW, IT RECEIVED A TIER I SCORE BY THE EXPERT SCIENTIFIC REVIEW IN GRANTS WORKING GROUP. AND DESPITE THE RECOMMENDATION THAT IT HAS EXCEPTIONAL MERIT AND WARRANTS FUNDING, IT'S NOT BEING RECOMMENDED BY THE CIRM TEAM FOR FUNDING. AND AS MENTIONED, ABOUT $4 MILLION ARE BEING LEFT ON THE TABLE DESPITE MULTIPLE BOARD MEMBERS ALSO MENTIONING THAT THIS IS A MERITORIOUS GRANT, AND THERE ARE MANY ASPECTS OF THIS THAT SHOULD BE ACTUALLY TAKEN UP.

SO THERE'S NO NEED TO CURTAIL THE GRANT. ONE COULD SIMPLY START THE FUNDING, AND THEN IN THE

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NEXT BUDGET CYCLE THERE WOULD BE MONEY SINCE THERE'S GOING TO BE REMIND-L AND THIS HAS BEEN RECOMMENDED FOR FUNDING.

I JUST WANT TO HIGHLIGHT SEVERAL REASONS WHY THIS SHOULD BE RECONSIDERED AND THIS IMPORTANT PROJECT FUNDED. A PREEMINENT GOAL OF PROP 14 IS TO DEDICATE 1.5 BILLION FOR DISEASES OF THE BRAIN SUCH AS SCHIZOPHRENIA AND AUTISM. AND OUR PROPOSAL, WHICH AIMS TO IDENTIFY THE DISEASE MECHANISMS UNDERLYING SCHIZOPHRENIA AND AUTISM BY USING STEM CELL MODELS, DIRECTLY ADDRESSES THIS URGENT PROP 14 PRIORITY.

ANOTHER IS THAT INCREASING THE REPRESENTATION OF GROUPS THAT ARE UNDERREPRESENTED IN BIOMEDICAL RESEARCH RECOGNIZES ANOTHER HIGH PRIORITY. OUR APPLICATION IS UNIQUE IN THIS WAY. WE'RE FOCUSING ON ONE OF THE MOST UNDERREPRESENTED POPULATIONS IN PSYCHIATRIC DISEASE RESEARCH, AFRICAN-AMERICANS. AND WE CREATE, AS THE REVIEWERS STATE, AN UNPRECEDENTED, UNIQUE RESOURCE FOR THE ENTIRE COMMUNITY.

THIRDLY, PROP 14 PRIORITIZES DIRECT PATIENT ENGAGEMENT AND OUTREACH ACTIVITIES THAT ENGAGE CALIFORNIA'S DIVERSE COMMUNITIES. OUR PROPOSAL IS THE ONLY ONE THAT BRINGS TOGETHER A

FOCUS ON COMMUNITY OUTREACH AND EDUCATION TO REMEDIATE THE LACK OF ADEQUATE INFORMATION THAT FAMILIES HAVE RECEIVED AS TO THE CRITICAL ROLE OF BIOMEDICAL RESEARCH IN THEIR FAMILY DISORDERS COMBINED WITH OUR WORLD-CLASS EXPERTISE IN NEUROPSYCHIATRIC DISORDER GENETICS, STEM CELL MODELING, AND AI TECHNOLOGIES TO EXECUTE ONE OF THE LARGEST INVESTIGATIONS TO DATE OF AUTISM AND SCHIZOPHRENIA DISEASE MECHANISMS AND UNDOUBTEDLY THE FIRST AND LARGEST OF ITS KIND IN AFRICAN-AMERICAN POPULATIONS.

A KEY WIDELY RECOGNIZED HURDLE IN STEM CELL RESEARCH IS SCALE AND THROUGHPUT. OUR ANALYSIS IS THAT THE LARGEST SCALE, LEVERAGING UNIQUE ADVANCED AUTOMATION PLATFORMS.

IN CLOSING, I THANK YOU FOR YOUR TIME AND ATTENTION. FOR MORE DETAILS AND OUR RESPONSE TO THE LARGELY UNFOUNDED CRITIQUES, PLEASE SEE OUR PUBLIC LETTER. GIVEN THE CLEAR ALLOCATION OF FUNDS FOR THESE CONDITIONS IN PROP 14, WE BELIEVE IT DOES NOT SERVE THE PURPOSE OF ADVANCING RESEARCH IN PSYCHIATRIC DISORDERS, ADDRESSING HEALTH DISPARITIES, AND ENGAGING OUR COMMUNITIES IN OUR MISSION TO WITHHOLD FUNDING AT THIS STAGE FROM THIS APPLICATION FOR WHICH FUNDING WAS RECOMMENDED. WHY

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WAIT? THANK YOU.

MS. MANDAC: THANK YOU, DR. GESHWIND. NEXT WE HAVE DR. HAUSSLER. YOUR TIME STARTS NOW.

DR. HAUSSLER: HELLO. YES, THANKS FOR THE OPPORTUNITY TO ADDRESS THE COMMITTEE. I THINK THIS ISSUE WITH THE AI IS A BIT OF A RED HERRING FOR THIS. WE ACTUALLY HAVE AN EXTRAORDINARILY STRONG TEAM AND EXTENSIVE BACKGROUND IN AI. I'VE BEEN IN THE FIELD FOR 40 YEARS, AND PROFESSOR MARIANOSCO (PHONETIC) IS ABSOLUTELY A STUNNING NEW GENIUS COMING OUT OF THE MIT PROGRAM IN THE LAST FIVE YEARS. SO WE HAVE A GREAT TEAM COMBINING MY LONG-TERM EXPERTISE AND HIS RECENT EXPERTISE.

I REALLY READ THE REVIEWERS AS KIND OF A MISGUIDED DON'T TRY ANYTHING NEW ATTITUDE. AND THIS IS EXACTLY THE ATTITUDE THAT HOLDS NIH BACK. WE HAVE TO BE BOLD AND CREATIVE, AND THAT MEANS EMPLOYING THE LATEST AI METHODS WHICH, AS WE HEARD EARLIER IN THE BOARD, ARE DEVELOPING FROM -- MEMBERS OF THE BOARD ARE DEVELOPING AT AN AMAZING RATE.

AI IS CHANGING THE WAY WE WORK. IT'S DEMONSTRATED BREAKTHROUGHS IN ALL SECTORS IT'S TOUCHED, INCLUDING MOLECULAR BIOLOGY WHERE, IN EFFECT, WAS RECENTLY AWARDED THE NOBEL PRIZE.

THROUGH THE MANY CONSORTIA IN WHICH WE ARE

LEADING DATA COORDINATION ANALYSIS HERE AT UCSC, WE ARE FOR THE FIRST TIME REACHING THE COMBINED CRITICAL DATA SIZE NEEDED TO UNLOCK EMERGING CAPABILITIES OF AI. DON'T LEAVE CIRM OUT OF THIS REVOLUTION. DON'T LEAVE AFRICAN-AMERICAN CELL LINES OUT OF THE PUSH TO HAVE AI HELP DECODE THE MECHANISTIC BASIS FOR GENOME AND DISEASE RELATIONSHIPS. THIS WILL SUCCEED.

I HAVE A VERY STRONG INTUITION ABOUT PUSHING THIS FORWARD NOW. I WOULD URGE YOU TO GET THIS STARTED. WE NEED TO GET THE CELL LINES AND WE NEED TO GET THE DATA GOING.

SO IF THERE'S MONEY LEFT ON THE TABLE, AS I UNDERSTAND THERE IS, THIS IS A HIGHLY, HIGHLY MERITORIOUS PROPOSAL. WE HAVE TO MAKE AFRICAN-AMERICAN GENOTYPES MATTER. AND WE'LL DO THAT BY A VERY SOPHISTICATED ANALYSIS OF A FIRE HOSE OF DATA THAT THE GESHWIND LAB IS ABLE TO PRODUCE FROM THESE CELLS, THESE EXTRAORDINARILY PRECIOUS CELLS. IT IS ABSOLUTELY ABSURD TO THROW THIS PROPOSAL UNDER THE BUS BECAUSE YOU DON'T BELIEVE THE AI WILL WORK.

MS. MANDAC: THANK YOU, DR. HAUSSLER. YOUR TIME IS UP. VITO, BACK TO YOU.

CHAIRMAN IMBASCIANI: THANK YOU, DOCTORS,

FOR YOUR WELL-THOUGHT OUT COMMENTS. ANY OTHER MEMBER OF THE PUBLIC WISH TO SPEAK? ANY BOARD MEMBER LIKE TO COMMENT? IF NOT, I'M GOING TO ASK SCOTT TO PROCEED TO A VOTE THEN.

MR. TOCHER: ALL RIGHT. THE MOTION IS TO ACCEPT THE TEAM RECOMMENDATION TO FUND APPLICATIONS 16337 AND 16345 AND NOT FUND APPLICATION 16400.

MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: YES.

MR. TOCHER: JUDY CHOU.

DR. CHOU: YES.

MR. TOCHER: LEONDRA CLARK-HARVEY.

DR. CLARK-HARVEY: YES.

MR. TOCHER: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MR. TOCHER: MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: YES.

MR. TOCHER: DAVID HIGGINS.

DR. HIGGINS: YES.

MR. TOCHER: VITO IMBASCIANI.

CHAIRMAN IMBASCIANI: YES.

MR. TOCHER: RICH LAJARA.

MR. LAJARA: YES.

MR. TOCHER: ADRIANA PADILLA.

DR. PADILLA: YES.

MR. TOCHER: JOE PANETTA.
MR. PANETTA: YES.
MR. TOCHER: MARV SOUTHARD.
DR. SOUTHARD: ABSTAIN.
MR. TOCHER: YAEL WYTE.
MS. WYTE: YES.
MR. TOCHER: KEVIN XU.
DR. XU: YES.
MR. TOCHER: THANK YOU VERY MUCH. THAT MOTION CARRIES. VITO.
DR. DULIEGE: MAY I MAKE A COMMENT?
CHAIRMAN IMBASCIANI: ANNE-MARIE, YES.
DR. DULIEGE: JUST I WANT TO SAY HOW MUCH I APPRECIATE THE TWO DOCTORS WHO PROVIDED THEIR TESTIMONY. AND TO MAKE SURE THAT THERE IS NO MISUNDERSTANDING, PARTICULARLY IN REGARDS TO THE LAST INTERVENTION, IT IS NOT THAT THE CIRM TEAM NOR THE ICOC FELT THAT THIS PROPOSAL LACKED MERIT. IN FACT, IF THERE WAS SO MUCH DISCUSSION IS BECAUSE WE BELIEVE THAT IT HAS MERIT. IT'S MOSTLY A MATTER OF BUDGET AND BUDGET CONSTRAINTS. SO JUST TO CLARIFY THAT POINT. THANK YOU.
CHAIRMAN IMBASCIANI: THANK YOU, ANNE-MARIE. WE CAN SKIP ITEM 14. AND NEXT ON THE AGENDA IS AGENDA ITEM NO. 11. THIS IS THE

CONSIDERATION OF THE APPLICATIONS THAT HAVE BEEN SUBMITTED IN RESPONSE TO CIRM'S TRANSLATIONAL PROGRAM ANNOUNCEMENTS TRAN1, 2, 3, AND 4. THIS WILL BE THE LAST TRANS CYCLE UNTIL A REPLACEMENT PROGRAM IS CREATED. GIL, YOU ARE GOING TO TAKE THE PRESENTATION?

DR. SAMBRANO: YES. THANK YOU VERY MUCH.

SO ONCE AGAIN, WE BEGIN WITH OUR MISSION, AND I WANT TO SHOW YOU JUST BRIEFLY WHERE THE TRAN PROGRAM SITS WITHIN OUR EXISTING PIPELINE OF PROGRAMS. THIS TAKES SINGLE-PRODUCT CANDIDATES THAT ARE PRODUCED POTENTIALLY THROUGH OUR DISCOVERY AWARDS, TAKES THEM THROUGH TRANSLATIONAL ACTIVITIES TO GET TO A PRE-IND MEETING OR AN EQUIVALENT IN ORDER TO PREPARE FOR CLINICAL ACTIVITIES.

EXCUSE ME FOR MY DRY THROAT.

SO THE TRANSLATIONAL PROGRAM OFFERS FOUR DIFFERENT TYPES OF OPPORTUNITIES FOR FOUR PRODUCT TYPES. SO THERE ARE THERAPEUTICS, THERE ARE -- I HAVE WATER. THANK YOU SO MUCH. I KEEP DRINKING IT. IT MARGINALLY HELPS, BUT THANK YOU. SO THERE'S THERAPEUTICS, DIAGNOSTICS, DEVICES, AND TOOLS. AND EACH OF THEM HAVE SORT OF THEIR OWN UNIQUE ACTIVITIES THAT THEY CONDUCT, AND I'LL SHOW YOU A LITTLE BIT MORE OF THAT IN A BIT. BUT JUST TO NOTE,

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IF THERE'S A NUMBER, SO FOR EXAMPLE, THE THERAPEUTIC HAS 27 APPLICATIONS THAT WERE CONSIDERED, WHICH IS WHERE WE GET THE MOST IN TERMS OF APPLICATIONS. THE DIAGNOSTIC, DEVICES, AND TOOLS HAVE MUCH FEWER JUST SO THAT YOU KNOW THE GENERAL LAY OF THE LAND, IF YOU WILL, FOR THE APPLICATIONS THAT WE GET.

NOW, EACH OF THESE DIFFERENT PRODUCT TYPES AS THEY ENTER INTO TRANSLATION HAVE TO BE AT A CERTAIN STAGE OF READINESS. AND SO ON, FOR EXAMPLE, WITH THE THERAPEUTICS, WE ARE LOOKING FOR CANDIDATES WHERE DISEASE MODIFYING ACTIVITY IS DEMONSTRATED THROUGH APPROPRIATE IN VITRO, IN VIVO MODELS. FOR THE OTHERS HAVING FEASIBILITY DEMONSTRATED THROUGH SOME TYPE OF PROTOTYPE BRINGS IT TO THE APPROPRIATE STAGE.

NOW, THERE ARE A VARIETY OF ACTIVITIES THAT ARE DEPENDENT ON, AGAIN, THE PRODUCT TYPE THAT ARE CONDUCTED DURING THE TRAN AWARD WITH THE GOAL OF EACH AT THE VERY END COMING TO A PRE-IND MEETING WITH THE FDA IF IT'S A THERAPEUTIC OR SOME OTHER PRE-SUBMEETING WITH THE FDA AS APPROPRIATE. OR IN THE CASE OF TOOLS, WHERE THEY REACH A POINT OF BEING ABLE TO TRANSFER THE DESIGN FOR MANUFACTURING AND DISTRIBUTION FOR OTHERS TO USE. AND THIS COULD BE FOR RESEARCH PURPOSES OR FOR CLINICAL PURPOSES.

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YOU WILL NOTE THAT EACH OF THE DIFFERENT PRODUCT TYPES ALSO HAS A DIFFERENT AWARD DURATION. SO FOR THE THERAPEUTIC, IT HAS UP TO 30 MONTHS, THE OTHERS UP TO 24 MONTHS TO COMPLETE THE ACTIVITIES. AND THE AWARD AMOUNTS ARE ALSO DIFFERENT AND TAILORED TO THE TYPES OF ACTIVITIES THAT EACH PRODUCT WOULD HAVE. SO 4 MILLION FOR A BIOLOGIC, WHICH TENDS TO BE THE MOST EXPENSIVE, DOWN TO A MILLION FOR THOSE THAT ARE PROPOSING A TOOL.

THE TRANSLATIONAL APPLICATIONS GO THROUGH THE REVIEW PROCESS, AGAIN, IN A VERY SIMILAR WAY AS I DESCRIBED BEFORE WITH THE THREE GENERAL STEPS. BUT IN CASES LIKE WE HAVE HAD IN THE LAST COUPLE OF TRAN CYCLES, WE END UP WITH MORE APPLICATIONS THAN THE GRANTS WORKING GROUP CAN HANDLE IN A SINGLE SESSION. SO WE DIVIDE THAT MERIT REVIEW INTO TWO PARTS.

WE HAVE A POSITIVE SELECTION COMPONENT WHERE THE GRANTS WORKING GROUP MEMBERS, INCLUDING OUR PATIENT ADVOCATE MEMBERS, MAKE SELECTIONS OF THE APPLICATIONS THAT THEY FEEL ARE LIKELY TO BE THE MOST IMPACTFUL AND RESPONSIVE. THOSE ADVANCE TO THE FULL MERIT REVIEW BY THE GRANTS WORKING GROUP.

AND SO IN THIS CASE WE RECEIVED 54 SUBMISSIONS FOR TRAN. FIFTY-THREE WERE ELIGIBLE.

AND THROUGH THE POSITIVE SELECTION PROCESS, 23 WERE SELECTED. AND THERE WERE NINE THAT BYPASSED THIS BECAUSE THEY HAD IN A PREVIOUS CYCLE A SCORE BETWEEN 80 AND 84. SO WE HAD A TOTAL OF 32 THAT WERE ADVANCED TO FULL GRANTS WORKING GROUP DISCUSSION AND SCORING. AND SO WE'RE BRINGING THOSE, THEN, RECOMMENDATIONS TO YOU.

THE COMPOSITION OF THE WORKING GROUP IS, AGAIN, SIMILAR TO THE COMPOSITION AS I DESCRIBED BEFORE FOR REMIND WITH OUR SCIENTIFIC MEMBERS, 15, WHO ARE SELECTED BASED ON EXPERTISE TO SCORE EACH APPLICATION, OUR GRANTS WORKING GROUP BOARD MEMBERS, AND SPECIALISTS AS NEEDED TO FILL IN KNOWLEDGE GAPS.

THE SCORING IN THIS CASE IS BASED ON A 1 TO 100 SCALE. SO SCORES FROM 85 TO A HUNDRED MEANS THAT THE APPLICATION SHOWS EXCEPTIONAL MERIT AND WARRANTS FUNDING. SCORES BELOW THAT ARE NOT RECOMMENDED FOR FUNDING.

THE CRITERIA THAT ARE UTILIZED BY THE GRANTS WORKING GROUP TO ARRIVE AT THEIR SCORE ARE BASED ON THESE FIVE QUESTIONS. DOES THE PROJECT HOLD THE NECESSARY SIGNIFICANCE AND POTENTIAL FOR IMPACT? IS THE RATIONALE SOUND? DOES IT HAVE A WELL-PLANNED PROJECT -- IS THE PROJECT WELL PLANNED AND DESIGNED? IS IT FEASIBLE? AND DOES IT UPHOLD

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THE PRINCIPLES OF DEI?

OKAY. SO THIS THE SUMMARY TABLE THE RECOMMENDATIONS FROM THE GRANTS WORKING GROUP. THERE WERE 17 APPLICATIONS OUT OF THIS BATCH THAT WERE DEEMED RECOMMENDED FOR FUNDING BASED ON THE SCORE OF HAVING 85 OR GREATER AND 15 THAT WERE NOT. THE TOTAL APPLICANT REQUEST OF THOSE 17 APPLICATIONS IS 78.2 OR SO MILLION. THE FUNDS AVAILABLE ARE 60 MILLION. SO, AGAIN, WE HAVE MORE APPLICATIONS THAT ARE RECOMMENDED THAN WE CAN FUND WITH THE BUDGET.

I WANT TO JUST MAKE A NOTE ABOUT MINORITY REPORTS. SOMETIMES YOU MAY OBSERVE THAT WE HAVE A MINORITY REPORT LINKED TO APPLICATIONS. THIS HAPPENS BASED ON WHETHER THE APPLICATION IS NOT RECOMMENDED FOR FUNDING, BUT HAS 35 PERCENT OR MORE MEMBERS THAT SCORE TO FUND THE APPLICATION. SO ANY APPLICATION THAT MEETS THOSE CRITERIA HAS A MINORITY REPORT INCLUDED. IN THIS PARTICULAR CYCLE THERE WERE NO APPLICATIONS THAT QUALIFIED FOR A MINORITY REPORT.

SO THE CIRM TEAM RECOMMENDATIONS, GIVEN THE BUDGET CONSTRAINTS, PUT US IN A PLACE WHERE WE CAN RECOMMEND UP TO 13. AND PART OF THE CONSIDERATION HERE WAS TO MAXIMIZE THE NUMBER OF APPLICATIONS BASED ON THE VARIABLE BUDGETS THAT ARE

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REQUESTED TO HAVE THE GREATEST NUMBER OF APPLICATIONS THAT CAN FIT WITHIN THAT BUDGET. SO THOSE 13 LEAD TO AN AMOUNT OF 57.5 MILLION, WHICH IS WELL WITHIN THE 60 MILLION THAT WE HAVE AVAILABLE TO US.

NOW, THE CIRM PROGRAMS TEAM, AND THIS IS LED BY DR. SHYAM PATEL, SO HE LEADS THE PRECLINICAL DEVELOPMENT TEAM, EXAMINED THE GRANTS WORKING GROUP RECOMMENDED APPLICATIONS. AND SO BASED PRIMARILY ON THE GWG SCORE OR THE RANK ORDER OF THE SCORE PUT TOGETHER THESE TEAM RECOMMENDATIONS. BUT THEY DID HAVE ADDITIONAL CONSIDERATIONS RELATED TO PORTFOLIO DIVERSIFICATION AND PROGRESSION. AND SO IF YOU WOULD LIKE ADDITIONAL COLOR ON THE RECOMMENDATIONS, I INVITE DR. SHYAM PATEL TO PROVIDE THAT.

BUT BEFORE WE GO THERE, I WANT TO HAVE US PUT UP THE EXCEL SPREADSHEET. AND THAT SHOWS THE LIST OF APPLICATIONS SO THAT YOU CAN SEE, HOPEFULLY, MORE CLEARLY WHICH APPLICATIONS ARE RECOMMENDED AND WHICH ONES ARE NOT. I ALSO WANT TO POINT OUT -- THEY'RE WORKING ON IT -- A COUPLE OF THINGS. AND MAYBE I'LL STATE IT.

SO YOU HAVE THESE MATERIALS IN FRONT OF YOU AS WELL IN HARD COPIES. THE LAST TRANSLATIONAL ROUND WE WERE IN SIMILAR SITUATION WHERE WE HAD MANY

MORE APPLICATIONS THAT WERE RECOMMENDED THAN WE COULD FUND. THERE WERE SIX APPLICATIONS THAT WERE ESSENTIALLY LEFT ON THE TABLE BECAUSE WE COULDN'T COVER THOSE. SO THOSE APPLICANTS REAPPLIED TO THIS CYCLE. THEY CAME BACK. ALL SIX ARE STILL IN THE TOP TIER, AND THOSE ARE ALL RECOMMENDED FOR FUNDING.

SO ESSENTIALLY THE RECOMMENDATIONS THAT WE HAVE WOULD INCLUDE THEM AS NOW THE GROUP THAT WOULD BE FUNDED. AND THEY ARE SHOWN AS AN ASTERISK WITH A YES UNDER THE RESUBMISSION COLUMN IN THAT TABLE.

CHAIRMAN IMBASCIANI: THANK YOU, GIL. I CERTAINLY HOPE WHEN YOU ASKED FOR MORE COLOR, YOU DIDN'T MEAN WITH RESPECT TO YOUR SPREADSHEET. TWO COLORS ARE ENOUGH.

OKAY. SO HERE WE HAVE IT, 17 RECOMMENDED BY THE GWG. THAT WAS NARROWED DOWN BY BUDGET CONSTRAINTS TO 13 THAT ARE FUNDED IN GREEN. AND -- I THOUGHT I HAD A REQUEST FOR A MOTION. I NEED A REQUEST -- A MOTION, EXCUSE ME, TO ACCEPT THE RECOMMENDATIONS OF THE TEAM.

DR. DULIEGE: IS IT TO ACCEPT THE RECOMMENDATION OF THE TEAM, OR IS IT TO ACCEPT THOSE THAT WERE NOT RECOMMENDED, GO THROUGH, AND THEN WE'LL BE ABLE TO KNOW THE RECOMMENDED. JUST TO UNDERSTAND --

CHAIRMAN IMBASCIANI: THANK YOU FOR THE CLARIFICATION QUESTION. I WAS LOOKING, GIVEN THE COMPLEXITY OF THIS SPREADSHEET FOR SORT OF A MORE OMNIBUS MOTION TO ACCEPT THE TOTAL RECOMMENDATION OF THE TEAM AS PRESENTED.

THERE IS JOE ON THE LINE. YES. OKAY. JOSEPH.

MR. PANETTA: THANK YOU. I'LL MAKE THAT MOTION.

CHAIRMAN IMBASCIANI: OKAY. THANK YOU VERY MUCH. DO I HAVE A SECOND?

MR. FISCHER-COLBRIE: SECOND.

CHAIRMAN IMBASCIANI: SECOND FROM MARK FISCHER-COLBRIE. THANK YOU.

SO THIS MOTION IS NOW OPEN TO DISCUSSION FROM MEMBERS OF THE BOARD. ANNE-MARIE, PLEASE.

DR. DULIEGE: THANK YOU AGAIN FOR THE VERY DETAILED EXPLANATION OR AT LEAST A VERY CLEAR EXPLANATION. AND WE UNDERSTAND TWO THINGS. GOOD NEWS IS THOSE WHO RESUBMITTED WERE FUNDED. THE IMPLICATION, THAT'S GREAT NEWS. THE SECOND, WE HAVE TO APPLY OUR JUDGMENT BECAUSE THE TOTAL ENVELOPE EXCEEDED THE BUDGET FOR THIS CYCLE.

IN THAT CONTEXT, IF YOU CAN HELP US BETTER UNDERSTAND WHY THERE WERE SOME YES THAT HAVE,

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THINKING ABOUT THE LAST ONE, THAT HAD A SCORE OF 85, OTHERS WITH A SCORE OF 87 WERE NOT RECOMMENDED FOR THE CIRM TEAM THAT WOULD HELP. THANK YOU.

DR. SAMBRANO: SO, SHYAM, IF YOU'D LIKE TO SPEAK TO THAT.

DR. PATEL: YEAH, CERTAINLY. SO OUR METHODOLOGY WAS TO FIRST RELY BY THE GWG RANKED SCORING. YOU CAN HEAR ME NOW. SO I'LL START AGAIN.

SO OUR METHODOLOGY WAS TO FIRST RELY ON THE GWG RANK SCORINGS. THE REVIEW TEAM PROVIDES TWO METRICS ON THAT, WHICH IS THE MEAN AND THE MEDIAN SCORE. SO IF YOU LOOK AT THAT LIST, UP UNTIL THE NINTH APPLICATION, I BELIEVE, THERE'S A CONSISTENT SCORE OF MEAN AND MEDIAN. ALL THOSE HAVE 87 OR HIGHER MEAN AND MEDIAN. AND SO THOSE WERE ONES THAT WE RECOMMENDED FOR FUNDING.

BELOW THAT THERE'S A MIX OF MEAN AND MEDIAN SCORES. AND SO THE FIRST THING WE DID WAS, FIRST, AGAIN, PRIORITIZE THE GWG SCORING, BUT THEN TO TAKE INTO CONSIDERATION PORTFOLIO DIVERSIFICATION.

AND TO GET TO ANNE-MARIE'S QUESTION AT HEART, THE LAST APPLICATION IS BEING RECOMMENDED FOR FUNDING, WHICH IS THE TRAN4 APPLICATION. BECAUSE AFTER WE HAD GOTTEN THROUGH THE TRAN1S, THAT WAS THE

ONE THAT COULDN'T BE FUNDED BECAUSE OF ITS BUDGET.

SO IF YOU GO TO -- YOU CAN'T SEE IT RIGHT NOW. BUT THE TRAN1 THAT IS THE LAST GREEN ONE, AFTER THAT THE REMAINING FUNDS ONLY ALLOW FOR FUNDING OF THAT TRAN4.

DR. DULIEGE: THANK YOU.

CHAIRMAN IMBASCIANI: ANYONE WANT TO MAKE A COMMENT? ANY HANDS RAISED?

MR. TOCHER: FOR BOARD MEMBERS.

MS. MANDAC: FOR PUBLIC?

CHAIRMAN IMBASCIANI: NOT YET.

MR. TOCHER: SORRY. WE'RE JUST SURVEYING FOR BOARD MEMBER COMMENT AT THE MOMENT. WE'LL GET TO PUBLIC COMMENT IN JUST A MOMENT.

CHAIRMAN IMBASCIANI: THANK YOU.

MR. TOCHER: IT DOESN'T APPEAR SO.

DR. DULIEGE: I DON'T MEAN TO ALWAYS BE THE ONE ASKING A QUESTION, BUT I WILL SAY APPRECIATE THIS. THERE'S ONE THING, THOUGH, AND, YES, THERE'S ACTUALLY VALUE IN DIVERSIFYING THE PORTFOLIO, INCLUDING FUNDING TRAN4. ON THE OTHER HAND, THIS ONE, TRAN4, HAD EIGHT YESES AND SEVEN NOS, WHICH MAKES IT VERY AVERAGE IN QUALITY, I THINK, OR FEASIBILITY PROPOSAL.

SO THAT'S WHERE IT BECOMES A LITTLE

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UNCLEAR TO ME. ARE WE FAVORING THIS TIME THE DIVERSITY OF PROPOSALS TO BE CLEAR VERSUS MAYBE, MAYBE THE QUALITY OR FEASIBILITY? AND THANK YOU FOR YOUR CLARIFICATION ON THIS ONE.

DR. PATEL: OF COURSE. YEAH. NO. SO THAT LAST ONE IS BECAUSE IT CAN BE FUNDED OUT OF THE ONES THAT WERE REMAINING. AND IT ALSO HAS BROAD POTENTIAL TO IMPACT EX VIVO GENETIC ENGINEERING OF CELLS. AND SO THAT IN A SENSE, IF THAT TOOL WERE TO PROGRESS WITHOUT THE FUNDING, IT COULD HAVE BROAD IMPACT ON OUR FIELD.

BUT -- SO THE WAY THAT THIS HAS BEEN APPLIED, JUST TO CLARIFY A LITTLE BIT MORE, IS THAT WE WENT THROUGH FIRST -- WE GOT TO THE 87, 87. THOSE ONES WERE ALL RECOMMENDED. THERE WAS ONE MORE RECOMMENDED, THEN THERE'S A REMAINING SET OF FUNDING THAT'S LEFT AFTER THAT. AND SO WE WENT LINE BY LINE WITH EACH OF THOSE AWARDS, THE TRAN1 APPLICATIONS THAT ARE THERE, AND THEN WE RECOMMENDED THE NEXT THREE AND NOT THE TWO BLOOD CANCER ONES FOR FUNDING. THAT WAS BASED ON PORTFOLIO DIVERSIFICATION AS WELL AS WITHIN OUR PORTFOLIO.

SO AFTER WE GET THROUGH THOSE THREE TRAN1S THAT ARE RECOMMENDED FOR FUNDING, THE ONLY ALLOWABLE REMAINING FUNDS WOULD ALLOW US TO FUND THAT TRAN4

BECAUSE IT WAS MERITORIOUS FROM THE GWG PERSPECTIVE, IT WAS ALLOWABLE FROM A FUNDING PERSPECTIVE, AND BECAUSE IT WAS A TRAN4 THAT COULD POTENTIALLY, AS A TECHNOLOGY PLATFORM, HAVE BROAD APPEAL, WE SUGGESTED TO RECOMMEND FOR FUNDING.

CHAIRMAN IMBASCIANI: FOLLOW UP, ANNE-MARIE? NO. OKAY. THAT WAS A VERY NICE EXPLANATION, SHYAM. THANK YOU VERY MUCH. I DON'T SEE ANY OTHER HANDS. MEMBERS OF THE PUBLIC WISH TO COMMENT ON THE MOTION?

MS. MANDAC: YES. THERE ARE HANDS RAISED. MEMBERS OF THE PUBLIC, IF YOU HAVE AUDIO ON ON YOUR END FOR THIS MEETING, IF YOU WOULD PLEASE TURN IT OFF. SO THERE IS ONE PERSON IN THE MEETING ROOM WHO WILL BE SPEAKING ON THIS APPLICATION AND FOUR CURRENTLY ON ZOOM.

I DO SEE ONE OF THE MEMBERS WITH A HAND RAISED I WAS EXPECTING FOR A CLINICAL PROGRAM. SO I JUST WANT TO REMIND PUBLIC MEMBERS WE ARE ON TRANSLATIONAL PROGRAM APPLICATIONS. SO COMMENTS SHOULD PERTAIN TO THE TRANSLATIONAL PROGRAM.

SO WE'LL START WITH THE FIRST SPEAKER, DANIEL LAU, WHO IS WITH US IN THE MEETING ROOM. DANIEL, IF YOU COULD APPROACH THE MICROPHONE. THERE IS A TIMER. YOU WILL HAVE THREE MINUTES. AS SOON

AS THE THREE MINUTES IS OVER, AUDIO WILL MUTE THE MICROPHONE. YOUR TIME STARTS NOW.

MR. LAU: MORNING.

CHAIRMAN IMBASCIANI: MORNING.

MR. LAU: WE ARE DAN AND MINNIE, AND WE ARE FROM SAN JOSE, CALIFORNIA. AND OUR SON MATTHEW HAS SANFILIPO SYNDROME TYPE B, ALSO KNOWN AS MPSIIIB.

I'M SPEAKING FOR APPLICATION 16907, WHICH WAS RECOMMENDED FOR FUNDING, BUT WAS LEFT OUT DUE TO BUDGET. TODAY MATTHEW IS HEALTHY, HAPPY, AND LIVELY. HE LOVES PLAYING HOT WHEELS WITH HIS OLDER BROTHER. HE LOVES BELTING OUT ELMO SONGS IN THE CAR. EVEN AT THREE YEARS OLD HE THINKS ABOUT OTHERS. HE SAVES HIS BROTHER SNACKS AND TREATS AND INVITES FRIENDS TO OUR HOUSE.

HE HAS A BRIGHT FUTURE, BUT IN A FEW YEARS HE'LL BE UNABLE TO SING ELMO SONGS, TALK, AND LAUGH WITH HIS BROTHER AND FRIENDS, OR RUN AROUND AND JUMP AROUND. HE WILL LOSE ALL MENTAL CAPACITIES AND WILL LIKELY DIE AS A TEENAGER.

SANFILIPO TYPE B IS 100 PERCENT FATAL. HE'S ALREADY SHOWING EARLY SYMPTOMS AND WILL FACE IRREVERSIBLE DECLINE WITHOUT TREATMENT.

ACCORDING TO THE UNIVERSITY OF FLORIDA,

THE ECONOMIC BURDEN FOR PATIENTS IS $8 MILLION. A CURE CAN REDUCE MASSIVE BURDENS ON THE HEALTHCARE SYSTEM.

THIS APPLICATION WAS UNANIMOUSLY VOTED 13 TO ZERO ON SCIENTIFIC RATIONALE, PLANNING AND DESIGN, FEASIBILITY, AND DEI PRINCIPLES.

ONE OUTLIER LIKELY AFFECTING THE SCORE WAS A CRUCIAL MISTAKE BY THE REVIEWERS IN CALCULATING THE INCIDENCE RATE. IT IS NOT 1 IN 1 MILLION, BUT IS ACTUALLY 1 IN 25,000 BASED ON PUBLISHED RESEARCH. IT IS OFTEN UNDERSCREENED AND UNDERCOUNTED.

ARTICLE 5, SECTION 4 OF THE GWG BYLAWS SAYS, "THE PRESIDENT SHOULD ALERT THE GWG AND THE APPLICATION REVIEW SUBCOMMITTEE TO MATTERS THAT HAVE BEEN FOUND TO BE INCORRECT IN THE REVIEW OF A GRANT APPLICATION." AMONG THE CLIN, TRAN, AND DISC BUDGETS, THERE IS A TOTAL OF $45 MILLION SITTING UNUSED AND POSSIBLY UP TO $125 MILLION VARIANCE BASED ON THE FY 24/25 NOVEMBER MEETING.

IS IT POSSIBLE FOR THE BOARD TO CONSIDER A REALLOCATION OF THE UNUSED BUDGETS TO FUND QUALIFYING TRAN1 APPLICATIONS LIKE THIS ONE ON SANFILIPO TYPE B? OR ALTERNATIVELY, COULD THE BOARD CONSIDER PARTIALLY FUNDING THE REMAINING 2.5 MILLION TRAN BUDGET IF THE TEAM BRINGS PRIVATE FUNDING?

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TIME IS NOT ON OUR SIDE, AND MATTHEW AND CHILDREN LIKE HIM DEPEND ON THIS. THANK YOU.

MS. MANDAC: THANK YOU SO MUCH, DAN, MINNIE, AND MATTHEW FOR JOINING US TODAY.

NEXT WE WILL PROCEED TO PEOPLE ON ZOOM. IT WILL BE DR. FLORENCE LAI FIRST FOLLOWED BY DR. KATE MASIUK. YOU WILL BOTH HAVE THREE MINUTES EACH. DR. LAI, YOU HAVE THE FLOOR.

DR. LAI: I'M FLORENCE LAI, A NEUROLOGIST AT MASS GENERAL HOSPITAL IN BOSTON, ASKING THAT THE BOARD VOTE TO FUND THE LIFESAVING STEM CELL GENE THERAPY FOR SANFILIPPO B, TRAN1-16907. THIS IS A FATAL BRAIN DISEASE THAT HAS BEEN COMPARED TO ALZHEIMER'S DISEASE BUT IN CHILDHOOD.

AS A RESEARCHER IN ALZHEIMER'S DISEASE MYSELF, I HAVE SEEN FIRSTHAND ITS DEVASTATING EFFECT ON HUNDREDS OF MY PATIENTS AND THEIR FAMILIES. A SIMILAR COGNITIVE AND PHYSICAL DETERIORATION IN SANFILIPPO B STARTS BEFORE AGE SIX WITH DEATH IN THE TEEN YEARS AND SOME AS YOUNG AS TEN. THIS WILL BE THE FATE OF MY GRANDSON MATTHEW, WHOM YOU'VE JUST SEEN, AND OTHER CHILDREN IF A CURE IS NOT DEVELOPED IN TIME.

I WAS SURPRISED TO LEARN THAT SANFILIPPO B IS NOT RARE. IT'S ABOUT 1 IN 25,000 LIVE BIRTHS

ACCORDING TO PUBLISHED NEWBORN SCREENING, WHICH IS THE GOLD STANDARD, NOT THE 1 IN 1 MILLION THAT REVIEWERS INCORRECTLY QUOTED, WHICH PROBABLY DECREASED THE SCORE. IN FACT, IT'S MUCH MORE COMMON THAN SEVEN OTHER GENETIC DISORDERS THAT RECEIVED GENEROUS CIRM FUNDING.

AND SANFILIPPO B IS ON PAR WITH SPINAL MUSCULAR ATROPHY, WHICH ALREADY HAS A GENETIC CURE AND HAS SAVED COUNTLESS LIVES. ALSO, THE PROPOSED STUDY WILL USE THE SAME TECHNOLOGY THAT HAS ALREADY PROVEN CURATIVE IN SANFILIPPO A, A VERY CLOSE VARIANT TO B. IN ANIMAL MODELS THE SANFILIPPO B SHOWS COMPLETE ERADICATION OF THE TOXIC HEPARAN SULFATE AND A REVERSAL TO NORMAL BRAIN -- NORMAL BEHAVIOR AND DEVELOPMENT.

THIS RESEARCH HAS THE COMMITTED INVOLVEMENT OF DRS. DONALD KOHN OF UCLA AND BRIAN BIGGER OF THE UK, LEADING WORLD EXPERTS IN THE LENTIVIRAL DELIVERY OF A NORMALIZED GENE FROM THE PATIENT'S OWN BLOOD AND IS A ONE-TIME CURE. THIS HAS GREAT IMPACT AND WILL BRING TREMENDOUS HOPE TO THOSE WITH SANFILIPPO B AND WILL SAVE MILLIONS OF DOLLARS IN LIFETIME HEALTHCARE COSTS.

THE NEED IS CRITICAL FOR THIS TRANSFORMATIVE GENE THERAPY TO CURE SANFILIPPO B.

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CHILDREN WITH SANFILIPPO B HAVE TICKING TIME BOMBS IN THE RELENTLESS GENERATION OF THIS CONDITION. THEREFORE, TIME IS OF THE ESSENCE. I URGE THE ICOC TO PUT FORTH A MOTION TO FUND THIS STUDY, PERHAPS EVEN DRAWING ON THE MILLIONS OF UNUSED DOLLARS AND OTHER BUDGETED CIRM INITIATIVES TO DO SO. I ASK FOR FLEXIBILITY THAT THIS COULD BE PARTIALLY FUNDED AND FAMILIES AND FOUNDATIONS COULD FUND THE REST. IT WOULD BE SO HELPFUL TO START THIS RIGHT AWAY.

PRECIOUS CHILDREN WITH SANFILIPO ARE DEPENDING ON YOU. THANK YOU VERY MUCH.

MS. MANDAC: THANK YOU SO MUCH, DR. LAI. NEXT UP WE HAVE DR. MASIUK TO BE FOLLOWED BY DR. YVONNE CHEN.

DR. MASIUK: HI. I'M KATE MASIUK. I'M A PI ON GRANT APPLICATION 16907, PROPOSING HEMATOPOIETIC STEM CELL GENE THERAPY FOR SANFILIPPO B. AS YOU'VE HEARD FROM THE FAMILY, SANFILIPPO B IS AN ABSOLUTELY DEVASTATING NEURODEGENERATIVE DISEASE THAT'S OFTEN REFERRED TO AS CHILDHOOD DEMENTIA.

THESE CHILDREN GO FROM BEING HAPPY AND HEALTHY KIDS TO SLOWLY LOSING THEIR DEVELOPMENTAL MILESTONES, EVENTUALLY BECOMING NONVERBAL, WHEELCHAIR BOUND, AND DYING IN THEIR TEENAGE YEARS. THERE ARE ZERO AVAILABLE TREATMENT OPTIONS.

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ONE REALLY IMPORTANT POINT THAT I WOULD LIKE TO MAKE ABOUT OUR PROPOSAL IS THAT IT HAS A VERY HIGH LIKELIHOOD OF DELIVERING AN ACTUAL CURE TO THESE PATIENTS. AND THAT'S BECAUSE, AS DR. LAI MENTIONED, THERE'S ALREADY A VERY SUCCESSFUL THERAPY THAT IS ALREADY IN THE CLINIC FOR THE CLOSELY RELATED SANFILIPPO A SYNDROME. THIS THERAPY WAS ALSO DEVELOPED IN THE LAB WITH OUR CO-I DR. BRIAN BIGGER, WHO IS ONE OF THE WORLD'S LEADING EXPERTS ON LYSOSOMAL STORAGE DISEASES. AND THIS USES AN ALMOST IDENTICAL APPROACH TO THE ONE THAT WE'RE PROPOSING, BUT IT JUST CHANGES THE AFFECTED GENE OF INTEREST.

AND WHAT WE'VE SEEN IN THE SANFILIPPO A TRIALS IS THAT CLEARING THE BRAIN OF THESE TOXIC HEPARAN SULFATE METABOLITES IN THESE PATIENTS CAN BE CURATIVE. AND I'M NOT TALKING ABOUT AN INCREMENTAL DIFFERENCE. BUT IN THIS TRIAL WE'VE SEEN THESE KIDS BECOME FULLY FUNCTIONAL, HEALTHY KIDS THAT CAN RIDE A SCOOTER OR EVEN ARGUE WITH THEIR PARENTS AT THEIR CLINIC VISITS.

WE'VE SHOWN SIMILARLY EXCELLENT MOUSE MODEL DATA IN OUR GRANT, THAT OUR THERAPY CAN ALSO COMPLETELY CLEAR BRAIN LEVELS OF HEPARAN SULFATE, SUGGESTING THAT OUR THERAPY CAN ALSO BE A CURE FOR THIS DISEASE.

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I'D ALSO LIKE TO JUST HIGHLIGHT THAT WE ACTUALLY HAVE THE TEAM IN PLACE TO EFFICIENTLY AND SUCCESSFULLY BRING THIS TO THE CLINIC. THE PRECLINICAL WORK OF THIS APPLICATION WILL BE DONE IN THE LAB OF DR. DONALD KOHN, WHO IS A CO-I ON THIS APPLICATION AND I'VE WORKED WITH FOR MANY YEARS. AND WE'VE SUCCESSFULLY BROUGHT SO MANY SIMILAR EX VIVO LENTIVIRAL VECTORS TO THE CLINIC.

WE KNOW AT THIS POINT -- WE HAVE ESSENTIALLY A REGULATORY PLAYBOOK TO IND. WE KNOW EXACTLY WHAT TOX STUDIES WOULD NEED TO BE DONE, EXACTLY WHAT PRECLINICAL DATA, EXACTLY HOW TO DO THE MANUFACTURING. I'M VERY CONFIDENT THAT WE CAN BRING THIS TO THE CLINIC AND HAVE IT BE EFFICACIOUS.

WE ALSO FEEL THAT THERE WERE SOME SERIOUS ERRORS MADE BY THE GRANTS WORKING GROUP IN OUR REVIEW. MOST IMPORTANTLY, THAT THEY CITED A 40-FOLD LOWER PREVALENCE FOR THIS DISEASE THAT IS ACTUALLY PUBLISHED FROM NEWBORN SCREENING WHEN POTENTIALLY UNDERESTIMATING THE IMPACT OF THIS THERAPY.

CIRM SET OUT IN 2020 VIA PROP 14 TO BRING NEW CURES TO THE CLINIC FOR NEURODEGENERATIVE DISEASES, AND THIS IS, I THINK, AN EXCELLENT OPPORTUNITY FOR CIRM TO INVEST IN A POTENTIAL CURE FOR THIS DISEASE. CIRM HAS BEEN HISTORICALLY A

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NATIONWIDE LEADER AND A BEACON OF HOPE FOR THESE FAMILIES IN RARE DISEASES. AND I ENCOURAGE THE BOARD TO CONSIDER FUNDING THIS PROPOSAL AND GIVE THESE FAMILIES HOPE.

MS. MANDAC: THANK YOU SO MUCH, DR. MASIUK. DR. CHEN, YOU'RE UP NEXT. AND AFTER THAT WILL BE THE PHONE NUMBER (619) 535-6876. SO ALL PRIOR SPEAKERS HAVE BEEN FOR 16907. DR. CHEN IS SPEAKING FOR 16912. DR. CHEN, YOU HAVE THE FLOOR.

DR. CHEN: THANK YOU VERY MUCH FOR THE OPPORTUNITY TO SPEAK. MY NAME IS YVONNE CHEN. I'M A PROFESSOR OF IMMUNOLOGY AND CHEMICAL ENGINEERING AT UCLA, AND I'M THE PI OF THE TRAN1-16912 APPLICATION THAT WILL DEVELOP A NEXT GENERATION CAR-T CELL THERAPY FOR GLIOBLASTOMA, WHICH IS BRAIN CANCER.

I WANT TO HIGHLIGHT FOUR POINTS IN SUPPORT OF OUR THERAPY. FIRST, RELATIVE TO CIRM'S MISSION TO DELIVER TRANSFORMATIVE TREATMENTS IN CALIFORNIA AND BEYOND, I WOULD LIKE TO EMPHASIZE THAT OUR TEAM HAS HAD A SUCCESSFUL TRACK RECORD IN TRANSLATING HOMEGROWN, CUTTING-EDGE THERAPY TO THE CLINIC. THE FIRST CAR-T CELL THERAPY DEVELOPED BY MY GROUP HAS YIELDED BEST-IN-CLASS SAFETY AND EFFICACY PROFILES IN PATIENTS WITH B-CELL LYMPHOMA, LEADING TO THE

FOUNDING OF A CALIFORNIA COMPANY THAT EMPLOYED OVER A HUNDRED SCIENTISTS. THIS THERAPY IS NOW ENTERING PHASE 2 CLINICAL TESTING.

FURTHERMORE, WITH OUR PREVIOUS TRAN1 FUNDING FOR BCMA-CS1 CAR-T CELL THERAPY, WE SUCCESSFULLY COMPLETED NOT JUST THE PRE-IND MEETING, BUT A FULL IND PACKAGE THAT HAS NOW BEEN APPROVED BY THE FDA FOR THE TRIAL TO PROCEED. THIS TRACK RECORD DEMONSTRATES OUR EXPERIENCE AND COMMITMENT TO DEVELOPING INNOVATIVE THERAPIES THAT CAN AND WILL BE EFFICIENTLY IMPLEMENTED IN THE CLINIC.

SECOND, THIS APPLICATION DESCRIBES A NOVEL AND AMBITIOUS APPROACH TO ADDRESS GLIOBLASTOMA, WHICH IS THE MOST COMMON TYPE OF PRIMARY BRAIN TUMORS THAT HAS NO EFFECTIVE TREATMENT OPTIONS. ONE MAJOR LIMITATION IS THAT GLIOBLASTOMA IS HIGHLY HETEROGENEOUS, MEANING WITHIN EVEN ONE PATIENT THE TUMOR CELLS CAN HAVE VERY DIFFERENT GENETIC AND PROTEIN EXPRESSION PROFILES. AND THAT HAS MADE IT VERY DIFFICULT FOR TARGETED THERAPIES, INCLUDING CAR-T CELL THERAPIES, TO ADDRESS.

HERE WE HAVE DEVELOPED A NEXT GENERATION THERAPY THAT CAN EFFECTIVELY ELIMINATE MULTIPLE MODELS OF ORTHOTOPIC BRAIN TUMORS INCLUDING ANTIGEN HETEROGENEOUS BRAIN TUMORS. VERY IMPORTANTLY,

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EFFICACY AND TOXICITY OFTEN FORM A DOUBLE-EDGED SWORD. AND IN OUR THERAPY WE HAVE DEVELOPED A WAY TO BOTH BOOST EFFICACY AND DIRECTLY ADDRESS POTENTIAL TOXICITIES THAT HAVE BEEN ASSOCIATED WITH CYTOKINE-ARMORED CAR-T CELL THERAPY. MORE DETAILS ON THIS CAN BE FOUND IN OUR WRITTEN PUBLIC COMMENTS.

THIRD, COST IS ALWAYS A SIGNIFICANT CONCERN WHEN IT COMES TO CELL-BASED THERAPIES. AND PART OF THIS PROJECT WILL BE FOCUSED ON DEVELOPING NOVEL MANUFACTURING PROTOCOLS THAT WILL SIGNIFICANTLY REDUCE THE TIME AND COST OF CAR-T CELL THERAPY WHICH WOULD BOTH BRING BENEFIT TO PATIENTS BY NOT ONLY REDUCING COST, BUT ALSO REDUCING THE AMOUNT OF TIME THEY HAVE TO WAIT BEFORE THEY CAN RECEIVE THERAPY.

FINALLY, I'D LIKE TO POINT OUT THAT THE GWG VOTES FOR OUR SPECIFIC REVIEW CATEGORIES, INCLUDING SIGNIFICANCE, RATIONALE, DESIGN, FEASIBILITY, AND DEI, WERE ALL CONSISTENTLY 11 TO 13 YESES OUT OF 13, INDICATING BROAD SUPPORT. SO WE HOPE THE AGS WILL CONSIDER SUPPORTING THIS VERY IMPORTANT PROJECT. AND WE THANK YOU FOR YOUR TIME.

MS. MANDAC: THANK YOU SO MUCH, DR. CHEN. YOUR TIME IS UP. I DO APOLOGIZE TO THE 619 NUMBER. I HAVE NOTICED THAT THERE IS ANOTHER SPEAKER FOR

16912. SO WE'D LIKE TO GO TO DR. LINDA LIAU FIRST AND THEN WE'LL GO TO THE PHONE NUMBER. SO, DR. LIAU, YOU HAVE THE FLOOR.

DR. LIAU: THANK YOU. CAN YOU ALL HEAR ME OKAY?

MS. MANDAC: YES.

DR. LIAU: HI. I'M LINDA LIAU, AND I AM A CHAIR IN THE DEPARTMENT OF NEUROSURGERY AT UCLA AND DIRECTOR OF THE UCLA BRAIN CANCER SPORE, WHICH IS ONE OF ONLY THREE CURRENTLY ACTIVE NCI-DESIGNATED SPORE PROGRAMS IN CALIFORNIA. I'M ALSO CO-INVESTIGATOR WITH DR. CHEN ON THIS APPLICATION ON NEXT GENERATION CAR-T THERAPY FOR GLIOBLASTOMA WHICH WAS DEEMED MERITORIOUS AND RECOMMENDED FOR FUNDING, BUT EXCLUDED BECAUSE OF BUDGET.

I'VE BEEN A PRACTICING NEUROSURGICAL ONCOLOGIST FOR 27 YEARS NOW; AND DESPITE SEEING AMAZING PROGRESS IN MANY OTHER CANCERS, BRAIN CANCER, PARTICULARLY GLIOBLASTOMA, STILL REMAINS ONE WITHOUT A CURE. AND THE AVERAGE PROGNOSIS OF THESE PATIENTS IS STILL LESS THAN TWO YEARS. THE MAJORITY OF THESE PATIENTS ARE YOUNG, BETWEEN THE AGES OF 30 TO 60, AND AT THE PRIME OF THEIR LIVES. AND IT IS TRULY HEARTBREAKING TO HAVE TO BREAK THIS DIAGNOSIS TO PATIENTS AND FAMILIES DAY AFTER DAY ONLY TO TELL

THEM THAT THERE'S NOT MUCH WE CAN DO BEYOND TRADITIONAL RADIATION AND CHEMOTHERAPY AND THEY REALLY ONLY HAVE TWO YEARS TO LIVE.

WE HAVE A VERY STRONG TRACK RECORD AT UCLA IN TRANSLATIONAL BRAIN CANCER RESEARCH AND BREAKING NOVEL CELLULAR THERAPIES AND IMMUNOTHERAPIES TO CLINICAL TRIALS, INCLUDING PERSONALIZED VACCINES AND OTHER CELLULAR THERAPIES SUCH AS THE ONE THAT WE'RE PROPOSING HERE.

I THINK ONE OF THE REASONS THAT WE CANNOT YET CURE BRAIN CANCER IS BECAUSE OF ITS LOCATION IN THE BRAIN AND THE WAY IT INVADES THE BRAIN.

GLIOBLASTOMA IS A CANCER THAT MALIGNANTLY INVADES THE BRAIN WITH FINGERS OF TUMOR CELLS THAT TRAVEL DEEP INTO AREAS OF THE BRAIN THAT WE CANNOT TAKE OUT SURGICALLY, WE CANNOT TARGET WITH RADIATION WITHOUT KILLING NORMAL BRAIN CELLS, AND WE CANNOT DELIVER DRUGS TO BECAUSE OF THE BLOOD-BRAIN BARRIER AND HETEROGENEITY. AS SUCH, NOVEL CELLULAR THERAPIES LIKE THIS APPROACH THAT WE ARE PROPOSING ARE LIKELY GOING TO BE THE MOST EFFECTIVE WAYS THAT WE CAN DELIVER THERAPEUTICS TO CANCER CELLS IN THE BRAIN.

I KNOW THAT CIRM HAS BUDGET CONSTRAINTS THAT PRECLUDES FUNDING OF ALL THE MERITORIOUS GRANTS

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BEING PROPOSED, BUT WE BELIEVE THAT THE IMPACT OF THIS PROJECT WILL FAR EXCEED THE INVESTMENT. GLIOBLASTOMA IS AN EXTREMELY DEVASTATING DISEASE, IMPACTING TENS OF THOUSANDS OF PATIENTS EACH YEAR WITH NO NEW FDA-APPROVED TREATMENTS IN ALMOST 20 YEARS. AS SUCH, PATIENTS ARE DESPERATELY SEEKING NEW, INNOVATIVE TREATMENTS. WE HAVE PATIENTS WEEKLY THAT ARE LEAVING CALIFORNIA THAT ARE TRAVELING ALL OVER THE COUNTRY AND TO OTHER COUNTRIES TO GET CELLULAR THERAPIES THAT ARE LIKELY LESS EFFICACIOUS THAN THE ONE THAT WE ARE DEVELOPING HERE.

CIRM HAS THE ABILITY TO MAKE CALIFORNIA TRULY A LEADER IN THIS FIELD.

MS. MANDAC: THANK YOU SO MUCH, DR. LIAU. THE NEXT SPEAKER WE HAVE IS A PHONE NUMBER, (619) 535-6876. IF YOU COULD PLEASE STATE YOUR NAME, WHAT APPLICATION YOU ARE SPEAKING ABOUT, AND THE FLOOR IS NOW YOURS.

DR. CONWAY: HELLO. MY NAME IS ANTHONY CONWAY, PI FOR APPLICATION TRAN 16956, HYPOIMMUNOGENIC IPSC-DERIVED TCR-NK CELLS FOR ONCOLOGY. THIS IS A RESUBMISSION OF APPLICATION TRAN1-16023 WHICH WAS ALREADY APPROVED FOR FUNDING BY THE ARS IN MAY 2024. I WAS THE PI FOR THAT APPLICATION AS WELL, BUT UNDER A DIFFERENT

FOR-PROFIT INSTITUTION. THAT INSTITUTION WAS UNFORTUNATELY NOT ABLE TO ACCEPT THE GRANT. BUT AS PER CIRM'S GUIDANCE, I RESUBMITTED THE APPLICATION UNDER A NEW NON-PROFIT INSTITUTION THAT WAS FOUNDED WITH THE SPECIFIC PURPOSE OF OPERATIONALIZING THE ACTIVITIES WITHIN THAT GRANT, STEM THERAPEUTICS, A CALIFORNIA-BASED COMPANY.

THIS NEW APPLICATION, RESUBMITTED APPLICATION, HAS THE SAME MILESTONES, DELIVERABLES, AND A STRENGTHENED TEAM, INCLUDING HOWARD FEDEROFF, A FORMER MEMBER OF THE GOVERNING BOARD OF CIRM WHERE YOU ALL ARE SITTING TODAY AS WELL AS TRENT SHARE (PHONETIC) OF MY COMPANY'S BOARD OF DIRECTORS. HE'S FULLY SUPPORTIVE OF THIS WORK AND COMMITTED TO THE SUCCESS OF STEM THERAPEUTICS AND PRO'S PROJECT. I WOULD REFER YOU TO HIS LETTER OF SUPPORT SUBMITTED WITHIN THE APPLICATION IN JULY OF 2024.

BOTH THE ORIGINAL SUBMISSION AND THE RESUBMISSION WERE RECOMMENDED FOR FUNDING BY THE GWG WITH THE ORIGINAL SCORING OF 90 AND THE MOST RECENT TIME SCORING 87. THE ORIGINALLY APPROVED FOR FUNDING $4.1 MILLION, TO THE BEST OF MY UNDERSTANDING, WAS NOT USED FOR ANY OTHER PROGRAM AND WENT BACK INTO THE CIRM GENERAL FUND.

THERE'S VARIOUS ADVANTAGES FOR THE THERAPY

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PROPOSED TO BE DEVELOPED WITHIN THIS PROPOSAL, INCLUDING COMPARED TO THE CURRENTLY APPROVED AUTOLOGOUS TCRT AND CAR-T THERAPIES FOR RELAPSED REFRACTORY MULTIPLE MYELOMA, INCLUDING THIS ALLOGENEIC TCR-NK, IS OFF THE SHELF, HAS LOW COST OF GOODS, HAS A BETTER SAFETY PROFILE THAN T CELL THERAPIES, HAS BETTER PATIENT ACCESSIBILITY, WHICH IS A KEY FOCUS FOR THE PROPOSED WORK AND THERAPY.

THE FACT THAT THIS THERAPY IS ALSO HYPOIMMUNOGENIC IPSC-DERIVED MEANS THAT IT'S RE-DOSABLE, HAS DECREASED DRUG PRODUCT HETEROGENEITY, INCREASED ANTITUMOR DURABILITY, AND FORMS THE BASIS OF A HIGHLY ENGINEERABLE IPSC CELL LINE THAT CAN BE USED FOR CREATING EVEN MORE HIGHLY ENGINEERED CELL THERAPIES FOR RECALCITRANT SOLID TUMORS.

THIS PATIENT POPULATION STILL HAS QUITE A HIGH UNMET MEDICAL NEED AND ACTUALLY A MULTIPLE MYELOMA PATIENT WILL BE SPEAKING BRIEFLY IN SUPPORT OF THIS PROPOSAL AS WELL. AND SO I WOULD HIGHLY ENCOURAGE THE BOARD TO RECONSIDER THE DECISION AND TO FUND THIS APPLICATION WHICH HAS ALREADY BEEN RECOMMENDED FOR FUNDING BY THE GWG TWICE AS WELL AS THE CIRM BOARD ONCE UNDER ITS ORIGINAL SUBMISSION UNDER THE DIFFERENT FOR-PROFIT INSTITUTION. THANK

YOU.

MS. MANDAC: THANK YOU SO MUCH, DR. CONWAY. SO THAT COMMENT WAS FOR 16956. MY SINCERE APOLOGIES TO THIS NEXT SPEAKER, GLENN O'NEILL FROM THE CURE SANFILIPPO FOUNDATION, FOR MISSING YOU IN THE FIRST TRANCHE WHEN I WAS GOING THROUGH 16907. THE FLOOR IS YOURS, GLENN.

DR. O'NEILL: THANK YOU. MY NAME IS GLENN O'NEILL, PRESIDENT OF CURE SANFILIPPO FOUNDATION. THANK YOU FOR THE OPPORTUNITY TO SPEAK ON BEHALF OF THE DIRE AND URGENT NEED FOR THE DEVELOPMENT OF A TRANSFORMATIVE TREATMENT FOR THE CHILDREN AND FAMILIES AFFECTED BY THE UTTERLY DEVASTATING NEURODEGENERATIVE DISEASE CALLED SANFILIPPO SYNDROME TYPE B.

MANY OF YOU HAVE LOVED ONES OR OTHER ADULTS YOU KNOW OF WHO HAVE BEEN AFFLICTED WITH FORMS OF DEMENTIA. AND I ASK YOU TO IMAGINE WHAT IT WOULD BE LIKE IF IT AFFECTED NOT AN ADULT WHO HAD A FULL LIFE, BUT A YOUNG CHILD WHOSE POTENTIAL IS ENTIRELY STOLEN.

CHILDREN WITH SANFILIPPO SYNDROME FACE A HEARTBREAKING PROGRESSION FOLLOWED BY FIRST INITIAL DEVELOPMENTAL DELAYS FOLLOWED BY A LOSS OF SPEECH, COGNITIVE ABILITY, MOTOR FUNCTION, CULMINATING IN

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DEATH OFTEN IN THE TEENAGE YEARS. CHILDREN GO FROM TALKING TO SCREAMING TO SILENCE, FROM EATING THEIR BIRTHDAY CAKE WITH JOY TO BEING FED THROUGH G-TUBE WHEN THEY CAN NO LONGER EAT BY MOUTH OR SWALLOW THEIR OWN SALIVA. THEY GO FROM RUNNING WILD AND FREE ON THE PLAYGROUND TO BEING UNABLE TO EVEN ROLL OVER IN BED. EACH SUCCESSIVE LOSS BRINGS A NEW WAVE OF GRIEF AND HARDSHIP.

AS THE PARENT OF A 15-YEAR-OLD DAUGHTER WITH SANFILIPPO SYNDROME, I CAN TESTIFY TO THIS REALITY. AND YOU CAN READ FROM OUR SUBMITTED COMMENTS AND NUMBER OF LETTERS FROM AFFECTED FAMILIES WHO ARE WAITING FOR THE STEM CELL THERAPY THAT THIS GRANT WOULD DEVELOP. AGAIN, THERE ARE NO TREATMENTS FOR THIS DISEASE WHICH IS DESTROYING OUR CHILDREN'S BRAINS AND BODIES.

THE GRANT 16907 IS DEVELOPING A HEMATOPOIETIC STEM CELL THERAPY WHICH WILL PROVIDE A LIFELONG RESTORATION OF ENZYMES TO CORRECT THE ROOT CAUSE OF THIS DISEASE, SAVING LIVES, AND REDUCING SOCIETAL COST BURDEN OF CARE. WE RESPECTFULLY ASK YOU TO RECONSIDER AND AWARD FUNDING TO THIS GRANT.

THIS RESEARCH HAS AN UNUSUALLY HIGH LIKELIHOOD OF SUCCESS TO PROCEED TO CLINICAL TRIAL, DEMONSTRATE EFFECTIVENESS, AND PROCEED THROUGH THE

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DRUG APPROVAL REGULATORY PATHWAY FOR THE FOLLOWING REASONS. FIRST, THE TEAM HAS A DEMONSTRATED TRACK RECORD AND EXPERTISE TO CONDUCT THIS WORK ACROSS PHASES OF DRUG DEVELOPMENT.

SECONDLY, EXTREMELY POSITIVE DATA IS COMING OUT OF THE CLINICAL TRIAL FOR A SISTER FORM OF SANFILIPPO TYPE A WHICH WAS DEVELOPED BY THE SAME SCIENTIST AS OUR CURRENT GRANT AND GIVES US CONFIDENCE THAT WE'LL SEE SIMILAR RESULTS. CHILDREN TREATED IN THAT TRIAL ARE ACHIEVING UNHEARD OF DEVELOPMENTAL GAINS AND EVEN TYPICAL NEURO DEVELOPMENT AT AN AGE WHERE THEY WOULD HAVE BEEN EXPECTED TO PROGRESS TO AN INFANTILE COGNITIVE LEVEL.

THIRD, THE SANFILIPPO COMMUNITY IS ROBUST AND ACTIVE IN ITS COMMUNICATION AND COLLABORATION WITH THE SCIENTIFIC COMMUNITY WHICH WILL ENABLE EFFICIENT AND FULL ENROLLMENT IN A FUTURE CLINICAL TRIAL.

AND LASTLY, THE FDA IS NOW ALIGNED WITH THE SCIENTIFIC COMMUNITY ON THE USE OF HEPARAN SULFATE AS A BIOMARKER WHICH ALLOWS US TO UTILIZE THE ACCELERATED APPROVAL PATHWAY, SPEEDING AND CLARIFYING THE PATH TOWARD POTENTIAL APPROVAL OF THIS THERAPY.

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AGAIN, WE IMPLORE THE BOARD RESPECTFULLY TO EXERCISE ITS DISCRETION AND ALLOCATE FUNDING TO THIS VERY MERITORIOUS AND NEEDED STEM CELL THERAPY IN LINE WITH ITS PRIORITIES, TRANSFORM THE BURDEN OF DEVASTATING NEUROLOGICAL DISEASE. THANK YOU FOR YOUR CONSIDERATION OF OUR SINCERE REQUEST. THANK YOU.

MS. MANDAC: THANK YOU SO MUCH, GLENN. MR. CHAIR, THERE ARE NO MORE COMMENTS.

CHAIRMAN IMBASCIANI: THANK YOU, CLAUDETTE, FOR MANAGING THAT. JUST A COMMENT ON THE PUBLIC COMMENT, IF YOU WILL. THE BOARD RECEIVED A VERY LARGE OUTPOURING OF COMMUNICATIONS FROM THE PUBLIC AND FROM THE SCIENTIFIC COMMUNITY. WE APPRECIATE THAT. IF I AM TYPICAL OF THE BOARD MEMBERS, I SPENT MUCH OF THE LAST WEEK READING THE DOZENS AND DOZENS OF THESE LETTERS IN THEIR ENTIRETY. I THANK THE SCIENTIFIC COMMUNITY FOR THEIR EXPLANATIONS AND SOMETIMES THEIR CRITIQUE OF OUR INTERNAL REVIEW. I THANK THE PARENTS AND FAMILY MEMBERS OF THE AFFECTED INDIVIDUALS FOR FLESHING OUT THE ENTIRE HUMAN IMPACT AND FROM OTHERS WHO JUST MADE VERY, VERY STRONG ARGUMENTS.

SO I'M SURE ALL BOARD MEMBERS TOOK STOCK OF EVERYTHING YOU SAID, AND WE APPRECIATE THE CARE

AND PASSION THAT YOU PUT INTO THAT EFFORT. THANK YOU.

YES, MARK.

MR. FISCHER-COLBRIE: JUST A FOLLOW-UP QUESTION, GIL. IF YOU COULD REMIND ME AGAIN WHAT STATUS THESE APPLICATIONS THAT HAVE BEEN VETTED FOR APPROVAL, BUT COULDN'T PROCEED FOR OTHER BUDGETARY REASONS. WHAT SORT OF TIMELINE DOES THAT FIT INTO GOING FORWARD?

DR. SAMBRANO: I'M SORRY. I JUST WANT TO MAKE SURE I UNDERSTAND THE QUESTION. YOU'RE SAYING AN OPPORTUNITY FOR THE FOUR THAT ARE NOT CURRENTLY RECOMMENDED BY THE CIRM TEAM TO HAVE AN ANOTHER OPPORTUNITY?

MR. FISCHER-COLBRIE: YES.

DR. SAMBRANO: SO THIS IS SIMILAR TO REMIND IN THE SENSE THAT WE'RE BRINGING NEW CONCEPTS TO THE BOARD IN MARCH THAT INCLUDES A MORE COMPREHENSIVE PROGRAM THAT'S RELATED TO TRANSLATIONAL ACTIVITIES PLUS OTHERS THAT WE WOULD EXPECT THESE COULD COME INTO ONCE THAT PROGRAM IS LAUNCHED. AND SO WE HAVE THE PROGRAMS TEAMS. AND, SHYAM, I DON'T KNOW IF YOU WANT TO SPEAK TO THAT IN A LITTLE MORE DETAIL.

DR. PATEL: YEAH. WE'RE WORKING AS FAST

AS WE CAN TO DEVELOP A NEW PROGRAM, PRECLINICAL DEVELOPMENT PROGRAM, WHICH IS GOING TO COMBINE ON THE TRANSLATIONAL AND IND-ENABLING ACTIVITIES, WHICH IS THE CLIN1, TO CREATE A FUNDING OPPORTUNITY THAT COULD POTENTIALLY ACCELERATE PROGRAMS FASTER THAN WE ARE DOING TO DATE.

DR. BARRETT: CAN I ASK A QUESTION ABOUT 16956, WHICH WAS A RESUBMISSION? AND IF I UNDERSTOOD THE SPEAKER CORRECTLY, THEY SAID THAT THE ORIGINAL --

THE REPORTER: MICROPHONE. THANK YOU.

DR. BARRETT: I WANTED TO SPEAK ABOUT 16956. AND IF I UNDERSTOOD THE SPEAKER CORRECTLY, WHO SPOKE ON BEHALF OF THIS APPLICATION, IT WAS ORIGINALLY AWARDED AND WAS NOT ABLE TO BE ACCEPTED BY THE PREVIOUS INSTITUTION. SO COULD SOMEBODY EXPLAIN WHY THAT WAS THE CASE?

DR. PATEL: YES. THIS SPEAKER HAS MENTIONED IT, SO I CAN SPEAK TO THAT AS WELL, WHICH IS THAT THIS APPLICATION WAS AWARDED FOR FUNDING IN THE PRIOR ROUND WHERE THERE WAS, AGAIN, A LIMITATION OF FUNDING. IT WAS ONE OF THE ONES THAT WAS AWARDED BY THE BOARD. DURING THE AWARD CONTRACTING, THE COMPANY DIVESTED FROM THAT PROGRAM, AND IT DIVESTED FROM A SIMILAR PROGRAM ALSO THAT IS ONGOING RIGHT

NOW AT MD ANDERSON WHERE THEY'RE IN CLINICAL TRIAL WITH A CORD BLOOD-DERIVED APPROACH FOR THE SAME INDICATION AND BASICALLY THE SAME TARGET.

AND SO THIS IS THE IPSC-DERIVED VERSION. AND SO IN THIS PARTICULAR INSTANCE, THEY FORMED A NON-PROFIT AND REAPPLIED FOR THIS ROUND.

DR. BARRETT: THANK YOU.

CHAIRMAN IMBASCIANI: FINAL OPPORTUNITY --

DR. DULIEGE: SO WE ARE CONTINUING TO EVALUATE, MAYBE SLIGHTLY STRUGGLE WITH THE SITUATION OF THESE FOUR APPLICATIONS BECAUSE, AGAIN, TO CLARIFY, THE CIRM TEAM BELIEVE THAT THEY HAVE MERIT TO BE FUNDED. IT'S REALLY A MATTER OF MOSTLY BUDGET, TO A LESSER EXTENT DIVERSITY OF THE APPLICATIONS.

AND SO EITHER SHYAM OR MAYBE J.T. AS WELL, WHAT ARE GOING TO BE THE MECHANISM WHERE WE CAN ADDRESS IT WHILE STILL RESPECTING THE NEED TO BE STRINGENT FROM A BUDGET PERSPECTIVE AND IN VIEW OF THE FACT THAT EVERYONE WHO IS AFFECTED BY THIS DISEASE DIRECTLY OR INDIRECTLY, I WOULD SAY MOSTLY THE FAMILY AND THE PATIENTS, REALLY FEEL A SENSE OF URGENCY. WE KEEP THAT IN MIND. IT'S AT THE FOREFRONT AND YET WE CANNOT ALWAYS ACT PURELY ON

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THAT FOR REASONS I JUST MENTIONED. SO THANKS.

WHAT WILL HAPPEN NEXT? THEY WILL BE ABLE TO RESUBMIT? WE NEVER CAN PROMISE ANYTHING AHEAD OF TIME, BUT THERE IS A FAIR LIKELIHOOD OF ACCEPTANCE AND, AGAIN, NO PROMISE THERE? CAN YOU CLARIFY THAT AND TIMELINES PLEASE?

DR. PATEL: SINCE THE PROGRAM IS UNDER DEVELOPMENT, I CAN'T COMMENT BEYOND THAT. BUT THAT PROGRAM IS DESIGNED TO COVER THE RANGE OF ACTIVITIES THAT ARE BEING PROPOSED HERE PLUS MORE TO GET TO THE IND.

DR. DULIEGE: TIMELINES PLEASE.

DR. PATEL: SO RIGHT NOW WHAT WE PUBLICLY DISCLOSED IS THAT WE WOULD TAKE THAT CONCEPT TO THE END OF MARCH BOARD MEETING. OUR NORMAL TIMELINES FOR ACTIVATING A PROGRAM ARE ROUGHLY TWO MONTHS AFTER THE CONCEPT APPROVAL. AND OUR INTENT IS TO MOVE AS QUICKLY AS POSSIBLE FOR A VERY COMPLICATED PROGRAM THAT WE'RE DEVELOPING HERE. THAT WOULD BE WHAT WE HAVE HISTORICALLY DONE.

DR. DULIEGE: JUST TO BE CLEAR AGAIN. AND THERE'S MANY IFS HERE. AND THESE ARE IMPORTANT.

DR. CANET-AVILES: WE NEED TO TAKE INTO ACCOUNT THAT IT WILL BE CONCOMITANT WITH THE OTHERS. BUT WE ARE TRYING TO REVAMP THE PROCESSES INTERNALLY

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SO WE CAN BE MORE EFFICIENT AND SWIFT. SO LATE SPRING IS WHEN THESE APPLICATIONS COULD BE AVAILABLE FOR APPLYING, WE HOPE, IF THE BOARD APPROVES IN MARCH. THAT'S THE GOAL. THAT'S WHAT WE ARE ALL WORKING TOWARDS.

CHAIRMAN IMBASCIANI: JUDY CHOU HAS A COMMENT AND THEN SUZANNE.

DR. CHOU: MAYBE IT'S A COMMENT AS WELL AS A QUESTION. THIS MAY NOT BE SPECIFICALLY ADDRESSING THE APPLICATION OF 16907, BUT I THINK I'D LIKE TO KNOW. FIRST OF ALL, I BELIEVE THE PREVALENCE OF THE DISEASE MUST BE A VERY IMPORTANT FACTOR WHEN WE CONSIDER THE GRANT. AND THEN IF THERE'S ARGUMENT ABOUT THIS DISCREPANCY THERE, HOW DO WE ADDRESS THAT? AND THAT'S A QUESTION TO THE TEAM. AND THIS WAS HOW DO WE TAKE THAT INTO CONSIDERATION?

DR. PATEL: CERTAINLY. WE LOOKED INTO THAT. AND THE REVIEWERS' COMMENTS, WHILE THERE ARE SOME TYPOS, THEY ARE BASED ON A PUBLICATION THAT WAS CITED BY THE APPLICANT AS WELL. IN THAT PUBLICATION THEY LOOKED AT A 20-YEAR TIME FRAME OF PATIENTS WHO HAD BEEN REGISTERED AS MEMBERS WHO HAVE THE VARIOUS TYPES OF MPS. IN THERE THEY FOUND THAT IN THE U.S. AS A WHOLE 40 PATIENTS WERE REGISTERED WITH HAVING MPSIIIB. NOW, THIS IS NOT NEWBORN SCREENING, BUT

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THAT'S WHAT THEY HAD PUT IN THERE OVER A 20-YEAR PERIOD.

SO BASED ON THAT, THEY CALCULATED INCIDENCE AND PREVALENCE, WHICH IS SOME OF WHICH WAS BEING CITED BY THE REVIEWERS.

THERE HAVE BEEN OTHER STUDIES DONE. FOR EXAMPLE, THERE HAS BEEN A TAIWANESE STUDY WHERE OVER A ONE-YEAR PERIOD THEY DID NEWBORN SCREENING FOR VARIOUS TYPES OF RARE DISEASES, INCLUDING MPSIIIB. IN THERE THEY SCREENED 70,000 PATIENTS AND FOUR PATIENTS HAD MPSIIIB, WHICH IS 1 IN 25,000 INCIDENCE THAT YOU HEAR.

CHAIRMAN IMBASCIANI: SUZANNE. I'M SORRY. YES.

DR. SANDMEYER: SO I REALIZED INFORMALLY COMMUNITIES ARE ALWAYS SUPPORTING AND POTENTIATING THE WORK THAT CIRM DOES. SO MY QUESTION IS IS THERE ANY FORMAL PRECEDENT FOR HAVING COMMUNITIES MATCH CIRM FUNDING?

DR. SAMBRANO: THERE'S PRECEDENT FOR MATCHING, BUT TYPICALLY THE WAY WE TREAT THE APPLICATIONS IS THERE'S A SPECIFIC BUDGET REQUEST FOR ACTIVITIES. AND SO WE EITHER FUND THE FULL AMOUNT OR WE DON'T. WE HAVEN'T HAD A PRECEDENT WHERE WE AWARD HALF OF WHAT'S REQUESTED AND ALLOW

FOR MATCHING, AT LEAST HISTORICALLY.

DR. SANDMEYER: IT COULD BE REQUIRED AS A MATCH.

DR. SAMBRANO: OH, AND WE HAVE. WE HAVE REQUIRED CO-FUNDING, WE HAVE REQUIRED MATCHING, AND SOME DO MATCH, BUT WE NORMALLY KNOW OF THAT AHEAD OF TIME, NOT IN THE COURSE OF, SAY, A DECISION HERE.

CHAIRMAN IMBASCIANI: ANNE-MARIE.

DR. DULIEGE: SO MAYBE LET'S FOLLOW UP ON THIS POINT. I'M NOT SUGGESTING THAT WE CHANGE THE WAY WE OPERATE TODAY FOR A VARIETY OF REASONS. BUT I'M CURIOUS TO HEAR FROM THE CIRM TEAM AT LARGE IF THIS SITUATION COULD BE CONSIDERED IN THE NEAR FUTURE. IS THERE MERIT TO THIS RECOMMENDATION THAT WAS MADE BY A MEMBER OF THE PUBLIC AS WELL AS BY ONE OF OUR COLLEAGUES HERE? AND THE CONSIDERATION IS THAT IF THE FUNDING -- THERE'S LIMITATION IN FUNDING, COULD IT BE CONSIDERED THAT WE WOULD APPLY PARTIAL FUNDING MATCHED BY PERSONAL FINANCES AND FUND-RAISING OTHERWISE? PLEASE COMMENT ON MERIT VERSUS DOWNSIDES, NOT FOR TODAY, BUT SHOULD WE CONSIDER IT IN THE FUTURE?

DR. THOMAS: SO I DO NOT THINK IT'S APPROPRIATE FOR TODAY SINCE WE'RE ON THE FLY, BUT IT IS SOMETHING THAT WE CAN DISCUSS INTERNALLY ON THE

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TEAM AND REVERT BACK.

DR. DULIEGE: THAT'S EXACTLY MY POINT. THANK YOU.

CHAIRMAN IMBASCIANI: IS THERE ANYONE --

MR. TOCHER: WE DO HAVE ADDITIONAL PUBLIC COMMENT THAT THE PERSON WHO WAS UNABLE TO REACH THROUGH THE FIRST ROUND. SO IF YOU'D LIKE TO MAKE THAT. CLAUDETTE.

DR. THOMAS: COULD YOU HOLD ON ONE SECOND? DR. PATEL HAS A RELEVANT POINT TO MAKE HERE.

DR. PATEL: THERE IS AN EXAMPLE OF AN APPLICANT COMMITTING CO-FUNDING AT THE BOARD MEETING AND THE AWARD BEING PARTIALLY FUNDED. WE'RE LOOKING INTO THE DETAILS OF THAT AT THE MOMENT.

CHAIRMAN IMBASCIANI: SHYAM, CAN YOU BE A LITTLE MORE SPECIFIC?

MR. TOCHER: I THINK HE SAID HE'S LOOKING INTO IT.

DR. PATEL: WE'RE LOOKING INTO IT IF THAT IS RELEVANT TO THE BOARD. IF YOU WANT US TO CONTINUE LOOKING INTO IT, WE CAN.

DR. CLARK-HARVEY: YEAH. I'M SORRY. BUT I APPRECIATE YOU TRUNCATING THAT, BUT WHAT DID YOU MEAN WHEN YOU FIRST SPOKE WHEN YOU SAID EXAMPLE? ARE YOU SAYING THAT THERE'S BEEN PRECEDENTS? ARE

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YOU SAYING THERE'S -- I THINK AS A BOARD MEMBER AND SINCE YOU DID STATE THAT OUT LOUD, I'D LIKE TO BETTER UNDERSTAND WHAT YOU WERE GETTING AT. THANK YOU.

DR. PATEL: CERTAINLY. SO I USED TO BE ON THE REVIEW TEAM, AND I RECALL AN EXAMPLE. THIS WAS DURING THE PROP 71 DAYS WHERE THERE WAS A SIMILAR ISSUE IN TERMS OF BUDGETS. AND THERE WAS AN APPLICANT WHO HAD AT THE BOARD MEETING PROPOSED TO CO-FUND AN APPLICATION. AND SO WE'RE LOOKING INTO THAT AND WHAT THE SITUATION THERE WAS.

DR. CLARK-HARVEY: I APPRECIATE YOUR TRANSPARENCY AND HISTORY.

UNIDENTIFIED SPEAKER: I APOLOGIZE FOR INTERRUPTING. THERE'S STILL PUBLIC COMMENT TO BE MADE ON 16956.

CHAIRMAN IMBASCIANI: IS THIS A MEMBER OF THE PUBLIC SPEAKING, CLAUDETTE?

MS. MANDAC: WE CAN GO UNMUTED, BUT YES. WE DO HAVE A HAND RAISED. WE DID EXPECT A PUBLIC COMMENT ON 16956. WE WERE NOTIFIED THROUGH A YOUTUBE CHAT. A REMINDER TO EVERYONE WATCHING, YOUTUBE IS ONLY FOR VIEWING. THERE IS NO ABILITY FOR US TO HEAR PUBLIC COMMENT THROUGH YOUTUBE. SO ALL THE DIAL-IN INFORMATION ON THE AGENDA IS TO JOIN

THE ZOOM WHERE YOU CAN MAKE PUBLIC COMMENT. THE AGENDA ALSO HAS INSTRUCTIONS IF YOU'RE DIALING IN BY PHONE. YOU MUST PRESS STAR NINE. FOR THOSE VIDEO CONFERENCING, YOU RAISE A HAND.

SO WE DO HAVE A PERSON WITH A HAND RAISED, (408) 773-3800. IF YOU COULD UNMUTE YOURSELF, YOU WILL HAVE THREE MINUTES. PLEASE MAKE SURE TO IDENTIFY YOURSELF AND THE APPLICATION NUMBER. YOUR TIME STARTS NOW.

DR. TSAI: ALL RIGHT. THANK YOU. MY NAME IS RUBY TSAI. I AM THE CEO AND FOUNDER OF APPLIED STEM CELL, A CALIFORNIA CORPORATION BASED IN MILPITAS, CALIFORNIA.

SO TODAY I'M HERE TO EXPRESS MY SUPPORT FOR ANTHONY CONWAY'S CIRM GRANT. THE GRANT NUMBER IS 16956. FOR THIS GRANT WE WILL PROVIDE GMP GRADE IPSC LINES AND GENE EDITING CAPABILITIES UNDER GMP TO SUPPORT HIS RESEARCH. AND WE ARE ALSO AN INDUSTRY PARTNER OF CIRM, PROVIDING GMP GRADE IPSC LINES TO CIRM GRANTEES.

SO OVER THE PAST 16 YEARS, WE HAVE EMPLOYED HUNDREDS OF INDIVIDUALS IN CALIFORNIA, CONTRIBUTING SIGNIFICANTLY TO THE STATE ECONOMY AND ALSO IN ADVANCING STEM CELLS AND REGENERATIVE MEDICINE RESEARCH.

SO ANTHONY'S PROPOSAL ADDRESSES NOT ONLY CRITICAL UNMET NEEDS OF ONCOLOGY PATIENTS, BUT ALSO BROADER CHALLENGES IN THE ALLOGENEIC CELL THERAPY SPACE. AS YOU KNOW, CURRENTLY MOST APPROVED CELL THERAPIES ARE AUTOLOGOUS, SUCH AS CAR-T CELL THERAPY, MEANING ONE PRODUCT IS MADE FOR ONE PATIENT. THESE THERAPIES ARE PROHIBITIVELY EXPENSIVE AND COSTING $450,000 PER DOSE AND UP TO SEVERAL MILLION DOLLARS FOR TWO OR THREE DOSES. EVEN MORE CONCERNING IS THAT LESS THAN 5 PERCENT OF PATIENTS HAVE ACCESS TO THESE TREATMENTS.

ANTHONY'S INNOVATIVE APPROACH STARTS WITH ONE SINGLE CELL LINE, WHICH IS IPSC OR INDUCED PLURIPOTENT STEM CELLS, ENGINEERED TO BE HYPOIMMUNOGENIC AND TARGET TO KILL CANCER CELLS. SO THIS ALLOWS THE RESULTING CELL PRODUCTS TO SERVE MANY PATIENTS IN A COST-EFFECTIVE MANNER. SO THESE OFF-THE-SHELF PRODUCTS CAN PROVIDE ON-DEMAND TREATMENT, ADDRESSING THE URGENT NEEDS OF PATIENTS WHO CANNOT AFFORD TO WAIT FOR CUSTOM THERAPIES. PATIENTS DON'T HAVE TIME TO WAIT AND PATIENTS NEED TO BE TREATED RIGHT NOW.

SO AT APPLIED STEM CELL WE ENVISION A WORLD WHERE EVERYONE HAS ACCESS TO THE MEDICINES THEY NEED BECAUSE THEY'RE AFFORDABLE, AVAILABLE, AND

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EFFECTIVE. SO ANTHONY'S PROJECT ALIGNS PERFECTLY WITH APPLIED STEM CELL'S VISION. SO ON BEHALF OF APPLIED STEM CELL'S BOARD OF DIRECTORS, I'M VERY PLEASED TO PROVIDE THIS NEW INFORMATION TO THE CIRM BOARD REGARDING CO-FUNDING.

APPLIED STEM CELL WILL PROVIDE A HALF MILLION DOLLARS EITHER IN CASH OR IN KIND TO SUPPORT ANTHONY'S PROPOSED CIRM PROJECT CONTINGENT UPON HIS SUCCESSFUL RECEIPT OF THE CIRM FUNDING FOR GRANT. THE NUMBER AGAIN IS 16956. AND AS ANTHONY ALREADY MENTIONED, THIS GRANT WAS FUNDED, AWARDED...

MS. MANDAC: THANK YOU SO MUCH. RUBY, THE TIME IS UP.

ARE THERE ANY OTHER MEMBERS OF THE PUBLIC WHO WOULD LIKE TO MAKE COMMENT ON TRANSLATIONAL PROGRAM APPLICATIONS? AS A REMINDER --

MR. STEVENSON: YEAH. MY NAME IS ALLEN STEVENSON.

MS. MANDAC: OKAY. IF YOU COULD IDENTIFY YOUR NAME, THE APPLICATION NUMBER, AND YOU HAVE THREE MINUTES.

MR. STEVENSON: MY NAME IS ALLEN STEVENSON. I'M SPEAKING ON APPLICATION 16956. MARCH 30TH OF 2016, I WAS SICK AND STANDING IN THE SHOWER AND I STARTED COUGHING. IT CAUSED MY L5

VERTEBRA TO COLLAPSE. I LATER FOUND OUT I HAD FRACTURES IN EVERY VERTEBRA, I HAD CLUSTERS OF FRACTURES IN MY RIBS AND STERNUM, AND I HAD LESIONS ALL OVER MY BODY. THEY TOLD MY WIFE THAT MY SKULL LOOKED LIKE SWISS CHEESE.

SO I'M A PATIENT. I'VE BEEN FIGHTING MULTIPLE MYELOMA SINCE 2016. I'M ALSO INVOLVED IN PATIENT ADVOCACY, AND I RUN A FACEBOOK GROUP WITH ABOUT 9,000 MEMBERS FROM AROUND THE WORLD.

I KNOW THE NEEDS OF THE MULTIPLE MYELOMA COMMUNITY GLOBALLY. THE CURRENT TREATMENTS CARRY MANY SIDE EFFECTS AND RISKS. THEY'RE EXTREMELY HARD ON PATIENTS. I DID BACK-TO-BACK STEM CELL TRANSPLANTS. ONE OF MY MEMBERS THAT DID CAR-T RECENTLY DID NOT RECOVER. THEY'RE ALSO FINANCIALLY VERY, VERY DIFFICULT BECAUSE OF THE NEED TO TRAVEL FOR THESE REALLY HARD TREATMENT OPTIONS.

SO I'M FULLY SUPPORTIVE OF THIS PROPOSED THERAPY. THERE'S JUST A NEED FOR MORE TREATMENT OPTIONS FOR THIS INCURABLE DISEASE. GOOGLE DOES NOT SAY NICE THINGS ABOUT MULTIPLE MYELOMA. AT THE TIME I WAS DIAGNOSED IT SAID THAT I HAD A FIVE-YEAR LIFE EXPECTANCY. THIS CAN HELP MEET THE NEEDS OF THE UNDERSERVED COMMUNITIES AND ALLOW PEOPLE IN RURAL AREAS A TREATMENT OPTION THAT DOES NOT REQUIRE THE

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EXPENSIVE TRAVEL TO THE MAJOR TREATMENT CENTERS. THANK YOU. ONE AGAIN, THIS IS ON APPLICATION 16956 THAT WAS PREVIOUSLY FUNDED FOR 4.1 MILLION.

MS. MANDAC: THANK YOU VERY MUCH, ALLEN.

CHAIRMAN IMBASCIANI: THANK YOU, SIR. I HAD ANNE-MARIE AND THEN J.T.

DR. DULIEGE: J.T., GO FIRST AND I'LL FOLLOW.

DR. THOMAS: SO WITH RESPECT TO THIS NOTION OF SUPPLYING CO-FUNDING, ET CETERA, THE UNDERSTANDING IN THE PREVIOUS INSTANCE WAS THERE WAS -- THE CO-FUNDING WAS BROUGHT TO THE TABLE IMMEDIATELY, WHICH IS A DIFFERENT SITUATION THAN WE'RE DEALING WITH HERE OF A SUGGESTION THAT CO-FUNDING COULD BE ATTAINED PHILANTHROPICALLY OR WHATEVER. THIS IS JUST NOT THE TIME TO TRY TO GET INTO THAT. AT SUCH TIME AS THE APPLICANT WOULD REAPPLY, WHICH WOULD, AS DR. PATEL SAID, BE SOMETIME IN THE SPRING, ASSUMING THE BOARD APPROVES THE PREDEVELOPMENT CONCEPT PLAN IN MARCH, TO THE EXTENT THAT THERE IS AN ELEMENT OF THIS THAT WOULD CONTEMPLATE A CO-FUNDING ADDITION TO THE CIRM GRANT, THAT CAN BE BROUGHT AND BE PART OF THE ANALYSIS OF THE GWG. THIS JUST ISN'T THE APPROPRIATE TIME TO GET INTO THAT.

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DR. DULIEGE: TOTALLY AGREE. AND I WANTED TO CLARIFY THAT IT IS FREQUENTLY THE CASE THAT AS BOARD MEMBERS WE HAVE TO WALK THIS FINE LINE BETWEEN CERTAINLY LISTENING TO THE COMMENTS FROM THE PUBLIC AND AT THE SAME TIME SUPPORTING THE CIRM TEAM'S QUALITY RECOMMENDATION THAT THEY HAVE MADE AFTER VERY CAREFULLY THINKING ABOUT ALL THE PROS AND CONS.

AND SO IN FOLLOW-UP TO WHAT YOU JUST SAID, J.T., I SUPPORT APPROVING THE RECOMMENDATION THAT HAS BEEN MADE WHILE MAYBE HAVING SOME FEEDBACK AT ANOTHER TIME IN DUE TIME ABOUT THESE OPPORTUNITIES FOR CO-FUNDING, WHEN, HOW, AND WHY, AND MAYBE WHETHER THEY ARE HELPFUL OR NOT. SO THANK YOU FOR THAT, J.T., FOR THIS CLARIFICATION.

CHAIRMAN IMBASCIANI: I SEE NO OTHER HAND FROM BOARD MEMBERS, AND THE PUBLIC, HAVING BEEN HEARD FROM, SCOTT, YOU MAY PROCEED TO A VOTE ON THE MOTION.

MR. TOCHER: SURE. AND I'LL REITERATE. THE MOTION IS TO ACCEPT THE TEAM'S RECOMMENDATION TO FUND THE 13 APPLICATIONS IDENTIFIED IN THE PRESENTATION AND TO NOT FUND THE REMAINING APPLICATIONS.

JUDY CHOU.

DR. CHOU: YES.

MR. TOCHER: LEONDRA CLARK-HARVEY.
DR. CLARK-HARVEY: YES.
MR. TOCHER: ANNE-MARIE DULIEGE.
DR. DULIEGE: YES.
MR. TOCHER: MARK FISCHER-COLBRIE.
MR. FISCHER-COLBRIE: YES.
MR. TOCHER: DAVID HIGGINS.
DR. HIGGINS: YES.
MR. TOCHER: VITO IMBASCIANI.
CHAIRMAN IMBASCIANI: YES.
MR. TOCHER: RICH LAJARA.
MR. LAJARA: YES.
MR. TOCHER: ADRIANA PADILLA.
DR. PADILLA: YES.
MR. TOCHER: JOE PANETTA.
MR. PANETTA: YES.
MR. TOCHER: MARV SOUTHARD.
DR. SOUTHARD: YES.
MR. TOCHER: YAEL WYTE.
MS. WYTE: YES.
MR. TOCHER: AND KEVIN XU.
DR. XU: YES.
MR. TOCHER: THANK YOU. THE MOTION CARRIES.
CHAIRMAN IMBASCIANI: THANKS, SCOTT.

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MR. TOCHER: AS A POINT OF ORDER, I THINK WHAT WE'LL DO IS, GIVEN WHERE WE ARE IN THE SCHEDULE, WE'LL BREAK FOR LUNCH NOW, RETURN IN 45 MINUTES. SO LET'S SAY 12:50, AND WE'LL PICK UP THE CLIN ROUND AT THAT TIME.

SORRY. MARK.

MR. FISCHER-COLBRIE: YEAH. I JUST WANT TO TAKE THE PERSPECTIVE THAT IN ADDITION TO OPPORTUNITIES FOR BEING ABLE TO REAPPLY FOR GRANTS, HOPEFULLY IN THE RELATIVELY NEAR TERM, BECAUSE OF THE CIRCUMSTANCES AROUND SOME OF THESE APPLICATIONS WHERE THEY HAD TO PASS THE REVIEW PROCESS, BUT THERE'S INSUFFICIENT BUDGETARY DOLLARS, THAT WE HAVE SORT OF AN INTERNAL FLAG OF SPECIAL CONSIDERATION, NOT IN TERMS OF THE PROCESSES, BUT JUST HIGHLIGHTING WHAT THE CIRCUMSTANCES ARE. SO AS WE GO THROUGH THE REVIEW PROCESSES, THAT THIS IS IN A CATEGORY THAT GETS HIGHLIGHTED.

DR. CLARK-HARVEY: I'M SO GRATEFUL FOR THE DISCUSSION AND FOR THE SHARING OF THE PUBLIC. AND I MIGHT I SAY THAT IS ONE OF THE MOST WELL-BEHAVED THREE-YEARS-OLDS I'VE SEEN. I HAVE TWO LITTLE BOYS AND THEY WOULD BE CLIMBING THE WALL. SO GOOD JOB, PARENTS. KEEP IT UP.

CHAIRMAN IMBASCIANI: I THINK WE CAN GO TO

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LUNCH AND RETURN AT 12:50. THANK YOU.

(A RECESS WAS TAKEN.)

CHAIRMAN IMBASCIANI: I'D LIKE TO COME BACK INTO SESSION FOR PART 2. HAYLEY, DON'T GO AWAY. WE'RE GOING TO DIRECT OUR ATTENTION NOW TO AGENDA ITEM NO. 10. THIS IS THE CONSIDERATION OF ALL THE APPLICATIONS THAT WERE SUBMITTED IN RESPONSE TO A CLINICAL PROGRAM ANNOUNCEMENT. AND THE PRESENTATION WILL BE MADE BY DR. HAYLEY LAM.

DR. LAM: GOOD AFTERNOON. IT'S MY PLEASURE TODAY TO PRESENT THE GRANTS WORKING GROUP RECOMMENDATIONS FOR THE CLINICAL PROGRAM.

AS ALWAYS, WE BEGIN WITH OUR MISSION, ACCELERATING WORLD-CLASS SCIENCE TO DELIVER TRANSFORMATIVE REGENERATIVE MEDICINE TREATMENTS IN AN EQUITABLE MANNER TO A DIVERSE CALIFORNIA AND WORLD.

CURRENT STATUS OF THE CLINICAL BUDGET WAS APPROVED JUST IN DECEMBER FOR THE REMAINDER OF THE FISCAL YEAR FOR 76.7 MILLION. THE CURRENT ASK TODAY FOR THESE SIX APPLICATIONS FOR YOUR CONSIDERATION ARE A TOTAL LITTLE OVER 50 MILLION.

A BIT ON THE CLINICAL PROCESS WHICH IS DIFFERENT FROM THE TRANSLATIONAL, WHICH WE DISCUSSED BEFORE LUNCH. THE SCORING SYSTEM IS A 1, 2, 3. A 1

IS A RECOMMENDATION FOR FUNDING. A 2 IS A DO NOT RECOMMEND AT THIS TIME, BUT PROVIDES THE APPLICANT TO SUBMIT WITH REVISIONS IN RESPONSE TO THE GRANTS WORKING GROUP. AND A SCORE OF 3 IS A DO NOT RECOMMEND, AND THE APPLICANT CANNOT RESUBMIT THE APPLICATION FOR SIX MONTHS.

THE SCIENTIFIC SCORING IS BASED ON THE FOLLOWING FIVE REVIEW CRITERIA. DOES THE PROJECT HOLD THE NECESSARY SIGNIFICANCE AND POTENTIAL FOR IMPACT? IS THE RATIONALE SOUND? DOES THE DATA SUPPORT THE PROJECT? IS THE PROJECT WELL-PLANNED AND DESIGNED? SO IS WHAT THEY'RE PLANNING TO DO WITH THE AWARD APPROPRIATE? IS THE PROJECT FEASIBLE? SO DOES IT HAVE A TEAM AND RESOURCES IN PLACE TO EXECUTE? AND DOES THE PROJECT UPHOLD PRINCIPLES OF DIVERSITY, EQUITY, AND INCLUSION? SO FOR THE CLINICAL PROGRAM, THIS FOCUSES PRIMARILY ON HEALTH EQUITY AND DIVERSITY.

THE CLINICAL PROGRAM ALSO HAS A DIVERSITY, EQUITY, AND INCLUSION SCORE. THE SCORE IS DETERMINED BY THE BOARD MEMBERS OF THE GRANTS WORKING GROUP, AND THE SCORING SYSTEM IS DIFFERENT. IT'S FROM A ZERO TO TEN. A TEN BEING AN OUTSTANDING RESPONSE.

THE CRITERIA FOR THE DEI SCORE ARE AROUND

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THE OVERARCHING CATEGORIES OF THE OVERALL COMMITMENT OF THE APPLICANT TO DEI, THE PROJECT PLANS THAT THEY PLAN TO EXECUTE IN SPECIFICITY WITH DEI, AND WITH REGARDS TO THE CULTURAL SENSITIVITY OF THE TEAM.

SO TOGETHER THE FORM OF THE REVIEW PANEL IS UP TO 15 MEMBERS OF THE ROLES, AND THESE FOLKS A SCIENTIFIC SCORE FOR ALL APPLICATIONS. THESE ARE EXPERTS IN DISEASE AREA, MANUFACTURING, PRODUCT DEVELOPMENT, AND REGULATORY. WE ALSO HAVE OUR GRANTS WORKING GROUP BOARD MEMBERS WHO ARE PATIENT ADVOCATE AND NURSE MEMBERS OF THE BOARD HERE, AND THEY PROVIDE THE DEI EVALUATION AND PROVIDE A DEI SCORE ON ALL APPLICATIONS AS WELL AS CAN PROVIDE A SUGGESTED SCIENTIFIC SCORE IF THEY SO CHOOSE.

FOR THE CLINICAL PANELS, WE ALSO HAVE SCIENTIFIC SPECIALISTS WHICH ARE NOT VOTING FOLKS THAT PROVIDE A SCIENTIFIC EVALUATION IN SPECIALIZED EXPERTISE AS NEEDED.

SO ON TO THE SPECIFIC APPLICATIONS FOR TODAY. THE FIRST ONE UP FOR DISCUSSION IS CLIN1-16103. THIS IS A GENE THERAPY FOR DOK7, CONGENITAL MYASTHENIC SYNDROME. THE APPLICANT IS REQUESTING JUST UNDER 3 MILLION WITH AROUND 700,000 IN CO-FUNDING FROM A CALIFORNIA ORGANIZATION. AND THIS IS TO COMPLETE THE TASKS NEEDED TO FILE AN IND.

A LITTLE BACKGROUND ABOUT THIS PROJECT. THE TARGETED INDICATION IS A GROUP OF RARE NEUROMUSCULAR DISORDERS THAT RESULTS IN MUSCLE WEAKNESS AND DUE TO THE FAULTY SIGNALING BETWEEN THE NERVE AND THE MUSCLE CELLS. THE SEVERITY OF THE DISEASE HAS A WIDE RANGE FROM NEEDING TO USE BREATHING AIDS TO A COMPLETE INABILITY TO MOVE.

CURRENT STANDARD OF CARE TREATS THE SYMPTOMS ONLY AND ARE ESSENTIALLY TREATMENTS THAT HELP WITH THE SIGNAL TRANSMISSION BETWEEN THE NEURONS AND MUSCLE CELLS. THESE TREATMENTS REQUIRE CHRONIC ADMINISTRATION, AND THE EFFECTIVENESS OF THEM CAN DECREASE OVER TIME. THE PROPOSED GENE THERAPY COULD POTENTIALLY BE A ONE-TIME TREATMENT.

CIRM DOES NOT HAVE ANY OTHER PORTFOLIO PROJECTS THAT ARE ACTIVE IN THE TRANSLATIONAL AND CLINICAL SPACE, AND THE APPLICANT HAS NOT PREVIOUSLY RECEIVED CIRM FUNDING.

THE RECOMMENDATION FROM THE GRANTS WORKING GROUP WAS A UNANIMOUS RECOMMENDATION TO FUND CLIN1-16103 WITH A DEI SCORE OF 8, AND THE CIRM TEAM RECOMMENDATION CONCURS WITH THE GRANTS WORKING GROUP FOR A RECOMMENDATION TO FUND THIS APPLICATION FOR 2.89 MILLION.

I'LL HAND IT OVER TO CHAIR IMBASCIANI.

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CHAIRMAN IMBASCIANI: THANK YOU, HAYLEY. ANY MEMBERS IN CONFLICT?

MR. TOCHER: YES.

CHAIRMAN IMBASCIANI: NO, NOT FOR THIS ONE.

MR. TOCHER: NO, THERE ARE. THERE'S FOUR.

CHAIRMAN IMBASCIANI: FOUR. I DIDN'T SEE THE SLIDE. OKAY. THERE YOU GO. THANK YOU. ALL RIGHT. CHAIR WILL ENTERTAIN A MOTION.

DR. SOUTHARD: MOVE APPROVAL.

CHAIRMAN IMBASCIANI: THANK YOU, MARV. CAN WE HAVE A SECOND PLEASE?

DR. DULIEGE: SECOND.

CHAIRMAN IMBASCIANI: ANNE-MARIE DULIEGE SECONDS. THE DISCUSSION OF THIS APPLICATION IS NOW OPEN FOR BOARD MEMBERS. IF THERE IS NONE, WE CAN ASK FOR THE PUBLIC'S COMMENTS.

MR. TOCHER: I DON'T SEE ANY. THERE ISN'T ANY.

CHAIRMAN IMBASCIANI: THERE ISN'T ANY. ALL RIGHT. SCOTT, THAT MEANS...

MR. TOCHER: JUST ONE SECOND. ALL RIGHT. THE MOTION IS TO FUND APPLICATION 16103.

MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: YES.

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MR. TOCHER: JUDY CHOU.
DR. CHOU: YES.
MR. TOCHER: LEONDRA CLARK-HARVEY.
DR. CLARK-HARVEY: YES.
MR. TOCHER: ANNE-MARIE DULIEGE.
DR. DULIEGE: YES.
MR. TOCHER: MARK FISCHER-COLBRIE.
MR. FISCHER-COLBRIE: YES.
MR. TOCHER: DAVID HIGGINS.
DR. HIGGINS: YES.
MR. TOCHER: VITO IMBASCIANI.
CHAIRMAN IMBASCIANI: YES.
MR. TOCHER: RICH LAJARA.
MR. LAJARA: YES.
MR. TOCHER: ADRIANA PADILLA.
DR. PADILLA: YES.
MR. TOCHER: JOE PANETTA. MARV SOUTHARD.
DR. SOUTHARD: YES.
MR. TOCHER: YAEL WYTE.
MS. WYTE: YES.
MR. TOCHER: AND KEVIN XU.
DR. XU: YES.
MR. TOCHER: I DON'T THINK JOE HAS REJOINED. JOE PANETTA, ARE YOU THERE? OKAY. IN ANY EVENT, THE MOTION CARRIES. THANK YOU.

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CHAIRMAN IMBASCIANI: I'M SORRY, SCOTT.

MR. TOCHER: YES. THE MOTION CARRIES.

CHAIRMAN IMBASCIANI: THANK YOU. OKAY. HAYLEY, YOU CAN PROCEED.

DR. LAM: ALL RIGHT. NEXT APPLICATION IS CLIN1-17165. THIS IS AGING THERAPY FOR SPINOCEREBELLAR ATAXIA TYPE III. THE APPLICANT IS REQUESTING AROUND 5.7 MILLION TO COMPLETE THE ACTIVITIES AND TO SUBMIT AN IND.

A LITTLE BIT OF BACKGROUND ABOUT THIS PROJECT. THIS IS A NEURODEGENERATIVE DISEASE CAUSED BY A MUTATION IN THE ATAXIN-3 GENE WHERE THERE ARE EXTRA REPEATS IN THE CODING REGION. THIS CAUSES PROBLEMS WITH THE ENZYME'S ABILITY TO INTERACT NORMALLY WITH OTHER PROTEINS AND CAUSES TOXIC ACCUMULATION OF THE ATAXIN.

SO THE SYMPTOMS USUALLY APPEAR IN MIDLIFE AND IMPACT SEVERAL AREAS, INCLUDING THE PATIENT GAIT, BALANCE, AND SPATIAL AWARENESS, AND EYE MOVEMENT. SO THIS IS A PROGRESSIVE DISEASE THAT IS EVENTUALLY FATAL.

THE CURRENT STANDARD OF CARE TREATS THE SYMPTOMS ONLY, MAINLY FOCUSED ON PAIN RELIEF AND THE ANTISPASTICITY DRUGS. THE PROPOSED TREATMENT IS AN ANTISENSE OLIGONUCLEOTIDE THAT ALLOWS THE CELLS TO

PRODUCE A FUNCTIONAL ATAXIN GENE PROTEIN BY SKIPPING OVER THE EXTRA REPEATED SECTION OF THE GENE.

CIRM DOES NOT HAVE ANY OTHER ACTIVE TRANSLATIONAL OR CLINICAL AWARDS IN THIS INDICATION, AND THE APPLICANT HAS NOT RECEIVED PRIOR CIRM FUNDING.

THE RECOMMENDATION FROM THE GRANTS WORKING GROUP WAS A UNANIMOUS RECOMMENDATION TO FUND CLIN1-17165 WITH A DEI SCORE OF 7, AND THE CIRM TEAM RECOMMENDATION CONCURS WITH THE GRANTS WORKING GROUP FOR A RECOMMENDATION TO FUND THIS APPLICATION FOR 5.69 MILLION. AGAIN, I HAND IT TO CHAIR IMBASCIANI.

CHAIRMAN IMBASCIANI: THANK YOU AGAIN, HAYLEY. CHAIR WOULD LIKE TO HEAR A MOTION TO FUND THE RECOMMENDED --

DR. DULIEGE: MOVE.

CHAIRMAN IMBASCIANI: OKAY. ANNE-MARIE.

DR. DULIEGE: YES. I MOVE THIS APPLICATION.

DR. SOUTHARD: SECOND.

CHAIRMAN IMBASCIANI: THANK YOU. ANY BOARD MEMBER WISH TO DISCUSS THIS APPLICATION, 17165? IF NOT, WE'LL OPEN IT TO THE PUBLIC.

MR. TOCHER: DOESN'T APPEAR TO BE ANY PUBLIC COMMENT.

CHAIRMAN IMBASCIANI: AND CLAUDETTE AGREES. OKAY. LET'S GO TO A VOTE THEN. THANK YOU.
MR. TOCHER: MARIA BONNEVILLE.
VICE CHAIR BONNEVILLE: YES.
MR. TOCHER: JUDY CHOU.
DR. CHOU: YES.
MR. TOCHER: LEONDRA CLARK-HARVEY.
DR. CLARK-HARVEY: YES.
MR. TOCHER: ANNE-MARIE DULIEGE.
DR. DULIEGE: YEAH.
MR. TOCHER: YSABEL DURON.
MS. DURON: YES.
MR. TOCHER: MARK FISCHER-COLBRIE.
MR. FISCHER-COLBRIE: YES.
MR. TOCHER: ELENA FLOWERS.
DR. FLOWERS: YES.
MR. TOCHER: DAVID HIGGINS.
DR. HIGGINS: YES.
MR. TOCHER: VITO IMBASCIANI.
CHAIRMAN IMBASCIANI: YES.
MR. TOCHER: RICH LAJARA.
MR. LAJARA: YES.
MR. TOCHER: CHRIS MIASKOWSKI.
DR. MIASKOWSKI: YES.
MR. TOCHER: ADRIANA PADILLA.

DR. PADILLA: YES.
MR. TOCHER: JOE PANETTA. MARV SOUTHARD.
DR. SOUTHARD: YES.
MR. TOCHER: YAEL WYTE.
MS. WYTE: YES.
MR. TOCHER: KEVIN XU.
DR. XU: YES.
MR. TOCHER: THANK YOU VERY MUCH. MR. CHAIR, THE MOTION CARRIES.
CHAIRMAN IMBASCIANI: THANK YOU AGAIN. IS THERE A QUESTION OR COMMENT -- NO. OKAY.
DR. DULIEGE: I KNOW WE'RE IN THE MIDDLE OF THIS REVIEW, BUT JUST EVEN NOW RIGHT AT THE END, IT MIGHT BE USEFUL FOR THE NEW BOARD MEMBERS AND FOR SOME OF US TO UNDERSTAND WHY THE PROCESS FOR THE CLIN REVIEW IS DIFFERENT FROM THE PROCESS THAT WE WENT THROUGH THIS MORNING, AND AT SOME POINT MAYBE TO DEAL AT ANOTHER TIME WHAT'S THE TOTAL BUDGET AND WHAT'S THE PART OF THE BUDGET FOR ANY WE'RE GOING TO FUND TODAY. WE CAN DO THAT AT THE END OF THE REVIEW IF THAT'S MORE PRACTICAL.
DR. LAM: I CAN JUST BRIEFLY ADDRESS THAT. SO AS I MENTIONED, I THINK THE THIRD SLIDE, THE TOTAL BUDGET ALLOCATION FROM THE BOARD FOR THE CLINICAL PROGRAM FOR THE FIRST HALF OF THIS YEAR IS

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76.7 MILLION, AND THE TOTAL ASK ACROSS ALL SIX APPLICATIONS THAT ARE BEING PRESENTED TODAY IS OVER ON 50 MILLION. I'LL PROVIDE SOME CONTEXT.

IN ADDITION TO THAT, I SUPPOSE COMPARED TO THE TRANSLATIONAL PROGRAMS, INSTEAD OF CONSIDERING THE APPLICATIONS AS A COMPLETE COHORT OF ALL APPLICATIONS THAT WERE SUBMITTED AND REVIEWED BY THE GRANTS WORKING GROUP, THE CLINICAL PROGRAM RIGHT NOW WE CONSIDER EACH APPLICATION INDIVIDUALLY AND MAKE FUNDING DECISIONS FOR EACH SEPARATE APPLICATION.

SO THE REASON BEING THAT AT THE GRANTS WORKING GROUP THAT IS ALSO HOW THE APPLICATIONS ARE CONSIDERED AS A SINGLE STANDALONE PROJECT AND NOT IN COMPARISON AGAINST OTHER CLINICAL SUBMISSIONS. I HOPE THAT'S HELPFUL.

DR. DULIEGE: YEAH. THAT'S HELPFUL. IT DOESN'T HELP US TO UNDERSTAND WHY IT'S A DIFFERENT PROCESS. AND I'M NOT TRYING TO SUGGEST THAT WE'RE CHANGING IT TODAY, BUT IT'S HELPFUL TO UNDERSTAND WHY IS IT DIFFERENT EVEN AT THE LEVEL OF THE GRANT WORKING GROUP, AND THEN WE CAN MOVE FORWARD.

DR. SAMBRANO: I'M HAPPY TO SPEAK TO IT IF YOU WANT RIGHT NOW. BUT WHEN WE STARTED THE CLINICAL PROGRAM, THE NUMBER OF APPLICATIONS THAT WE WOULD GET PER CYCLE, SO WE WERE DOING 12 CYCLES PER

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YEAR, VARIED. SOMETIMES IT WAS JUST A SINGLE APPLICATION. AS A RESULT, THERE WAS NOTHING TO COMPARE IT TO. AND SO WE ESTABLISHED A SCORING SYSTEM OF 1, 2, 3 AND A PROCESS THAT LOOKED AT THEM INDIVIDUALLY.

SO IN THE CASE WHERE WE GOT ONE, WE COULD REVIEW THAT SINGLE ONE. IF WE GOT THREE, THERE WAS NO NEED TO COMPARE THEM. EACH ONE WAS TREATED AS IF IT WAS ALONE IN THE CYCLE. AND SO THAT HAS CONTINUED ALTHOUGH NOW WE'VE REACHED A POINT WHERE WE'RE GETTING MORE APPLICATIONS. SO IN RETHINKING THE CLINICAL PROGRAM, THAT'S PART OF WHAT WE'RE THINKING ABOUT.

DR. DULIEGE: THANK YOU.

CHAIRMAN IMBASCIANI: THANKS, GIL. THAT WAS VERY HELPFUL. SUZANNE.

DR. SANDMEYER: ALSO, I GUESS TO FOLLOW UP, IT SEEMS LIKE A FACTOR IS THAT THERE IS LESS ALLOCATED NOW TO FUND THE ONES THAT WE CONSIDER THAN THE MAXIMUM POSSIBLE. AND SO AT SOME POINT THOSE TWO NUMBERS MIGHT COME TOGETHER, AND THEN WE MIGHT WANT TO CHANGE THE PROTOCOL BEFORE THAT OCCURS IF THAT MAKES SENSE.

CHAIRMAN IMBASCIANI: OKAY. NO OTHER QUESTIONS. LET'S MOVE ON THEN.

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DR. LAM: THE NEXT APPLICATION IS CLIN2-14796. THESE ARE THE CONFLICTS OF INTEREST.

THIS APPLICATION IS A PROGRAM PROPOSING A STEM CELL THERAPY TO INDUCE DELAYED TOLERANCE AFTER A KIDNEY TRANSPLANT. THE APPLICANT IS REQUESTING OVER 7 MILLION TO COMPLETE A PHASE 1 CLINICAL TRIAL.

A LITTLE BIT OF BACKGROUND ON KIDNEY TRANSPLANTS. KIDNEY FAILURE HAPPENS WHEN THE KIDNEYS CAN NO LONGER FILTER AND CLEAN THE BLOOD AS NEEDED. AND PEOPLE WITH KIDNEY FAILURE REQUIRE LONG-TERM DIALYSIS OR A KIDNEY TRANSPLANT.

CURRENTLY THESE TRANSPLANT RECIPIENTS REQUIRE LIFELONG IMMUNOSUPPRESSIVE DRUGS TO PREVENT THE REJECTION OF THE DONOR ORGAN. AND THESE IMMUNOSUPPRESSION DRUGS INCREASE THE RISK OF INFECTION, CANCER, DIABETES, AND HEART DISEASE.

THE GOAL OF THE CURRENT PROJECT IS TO DELIVER AND INFUSION OF THE DONOR BLOOD STEM AND PROGENITOR CELLS AFTER THE KIDNEY TRANSPLANT AND WITHDRAW IMMUNOSUPPRESSIVE DRUGS. THIS WOULD ULTIMATELY IMPROVE THE QUALITY OF THE LIFE OF THE PATIENTS AND PROLONG THE FUNCTIONALITY OF THE DONOR KIDNEY.

THE CIRM PORTFOLIO HAS SEVERAL ACTIVE KIDNEY TRANSPLANT PROJECTS AT DIFFERENT STAGES OF

CLINICAL TRIALS. ONE IS A PHASE 3 TRIAL THAT USES A SIMILAR APPROACH IN A SIMILAR TARGET POPULATION, WHICH IS HLA-MATCHED KIDNEY TRANSPLANT RECIPIENTS EXCEPT THAT THE STEM CELL TRANSPLANT IS COMBINED AT THE SAME TIME AS THE KIDNEY TRANSPLANT.

THE OTHER THREE PROJECTS ARE EARLY PHASE CLINICAL TRIALS FOR HLA-MISMATCHED KIDNEY RECIPIENTS, INCLUDING ONE FOR PEDIATRIC PATIENTS WITH GENETIC DISEASES. AND THEY HAVE SIMILAR APPROACHES AS WELL EXCEPT WITH DIFFERENT COMBINATIONS OF CELL TRANSPLANTS OR DIFFERENT TIMING OF THE KIDNEY OR THE STEM CELL TRANSPLANT ITSELF.

THIS PARTICULAR APPLICANT HAS NOT RECEIVED PRIOR CIRM FUNDING, AND THE RECOMMENDATION FROM THE GRANTS WORKING GROUP WAS A UNANIMOUS RECOMMENDATION TO FUND CLIN2-14796 WITH A DEI SCORE OF 8. AND THE CIRM TEAM RECOMMENDATION CONCURS WITH THE GRANTS WORKING GROUP RECOMMENDATION TO FUND THIS APPLICATION FOR 7.34 MILLION. PASS TO CHAIR IMBASCIANI.

CHAIRMAN IMBASCIANI: ANY QUESTIONS OF HAYLEY --

VICE CHAIR BONNEVILLE: I ACTUALLY HAVE A QUESTION BEFORE A MOTION IS THE TABLE. I KNOW -- I'M GOING TO CHANNEL STEVE JUELSGAARD, WHICH WE'VE

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MENTIONED HIM SEVERAL TIMES TODAY. I DON'T KNOW IF HE'D BE HAPPY ABOUT THAT OR NOT. I WANTED TO UNDERSTAND HOW THIS PROPOSAL FITS IN WITH OTHER THINGS THAT WE'VE FUNDED IN THIS AREA. I KNOW WE FUNDED A LOT IN THIS AREA. SO I JUST WANT TO UNDERSTAND FROM A BIGGER PICTURE OF OUR PORTFOLIO HOW IT WORKS --

DR. LAM: THANK YOU. I'M GOING TO ASK DR. CANET-AVILES TO SPEAK TO THIS.

DR. CANET-AVILES: THANK YOU, MADAME VICE CHAIR.

SO AS DR. LAM WAS MENTIONING, WE HAVE THREE OTHER PROJECTS. AND ONE OF THEM, THE ONE THAT WE CAN SEE ON THE TOP, HAS FUNDED A SUCCESSFUL PHASE 3 CLINICAL TRIAL WHICH IS ALSO A MATCH TOLERANCE PROGRAM. AND AS HAYLEY WAS SAYING, THE ONLY DIFFERENCE IS THE DELAY. AND THE COMPANY HAVING COMPLETED THIS PHASE 3 CLINICAL TRIAL HAS ACTUALLY BEEN LOOKING FOR PARTICIPANTS TO COMMERCIALIZE. THEY DISSOLVED THE COMPANY, SUSPENDING OPERATIONS WHILE THEY ARE TRYING TO FIND A PARTNER TO COMMERCIALIZE.

THE OTHER TWO PROGRAMS, PERHAPS THEY CAN HAVE AN EFFECT IN A LARGER POPULATION BECAUSE THOSE TWO ARE FOR MISMATCHED APPROACHES. SO THAT'S THE

COMPARISON.

VICE CHAIR BONNEVILLE: THANK YOU.

DR. CANET-AVILES: THANK YOU.

CHAIRMAN IMBASCIANI: OKAY. I DON'T SEE ANY OTHER QUESTIONS, SO WE CAN ENTERTAIN A MOTION.

DR. DULIEGE: I MOVE TO APPROVE THIS APPLICATION.

CHAIRMAN IMBASCIANI: TO APPROVE THE APPLICATION.

DR. SOUTHARD: SECOND.

CHAIRMAN IMBASCIANI: IT'S MOVED AND SECONDED. WE CAN PROCEED TO DISCUSSION AMONG THE BOARD MEMBERS. NOTHING. OKAY. IS THERE ANY COMMENT FROM THE PUBLIC? THERE IS NOT. ALL RIGHT, SCOTT. YOU CAN PROCEED TO THE VOTE.

MR. TOCHER: MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: YES.

MR. TOCHER: JUDY CHOU.

DR. CHOU: YES.

MR. TOCHER: LEONDRA CLARK-HARVEY.

DR. CLARK-HARVEY: YES.

MR. TOCHER: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MR. TOCHER: YSABEL DURON.

MS. DURON: YES.

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MR. TOCHER: MARK FISCHER-COLBRIE.
MR. FISCHER-COLBRIE: YES.
MR. TOCHER: ELENA FLOWERS.
DR. FLOWERS: YES.
MR. TOCHER: DAVID HIGGINS.
DR. HIGGINS: YES.
MR. TOCHER: VITO IMBASCIANI.
CHAIRMAN IMBASCIANI: YES.
MR. TOCHER: RICH LAJARA.
MR. LAJARA: YES.
MR. TOCHER: CHRIS MIASKOWSKI.
DR. MIASKOWSKI: YES.
MR. TOCHER: ADRIANA PADILLA.
DR. PADILLA: YES.
MR. TOCHER: MARV SOUTHARD.
DR. SOUTHARD: YES.
MR. TOCHER: YAEL WYTE.
MS. WYTE: YES.
MR. TOCHER: KEVIN XU.
DR. XU: YES.
MR. TOCHER: JUST CHECKING FOR JOE PANETTA. NO, I DON'T BELIEVE HE IS ON. GREAT. THANK YOU VERY MUCH. THE MOTION CARRIES.
CHAIRMAN IMBASCIANI: THAT'S VERY GOOD. ALL RIGHT, HAYLEY. WE'RE ON 17081.

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DR. LAM: ALL RIGHT. CLIN2-17081. THIS IS AN APPLICATION FOR A NEURAL STEM CELL THERAPY FOR HUNTINGTON'S DISEASE. THE APPLICANT IS REQUESTING JUST UNDER 12 MILLION TO COMPLETE THE PHASE 1 B/2A CLINICAL TRIAL.

A LITTLE BIT OF CLINICAL BACKGROUND ABOUT THIS ONE. HUNTINGTON'S DISEASE IS AN INHERITED NEURODEGENERATIVE DISEASE WHERE THERE IS MUTATIONS IN THE HUNTINGTIN GENE. SO THE MOVEMENT, COGNITION, AND PSYCHIATRIC SYMPTOMS USUALLY ARISE THE MIDLIFE AND PROGRESS OVER THE COURSE OF 10 TO 20 YEARS. EVENTUALLY THE DISEASE IS FATAL AND ALSO HAS A HIGH BURDEN ON FAMILIES.

THERE'S CURRENTLY NO APPROVED THERAPIES THAT SLOW OR PREVENT PROGRESSION OF THE UNDERLYING DISEASE. THE GOAL OF THE PROPOSED PROJECT IS TO PROVIDE NEURAL STEM CELLS TO SUPPORT THE SURVIVAL AND CONNECTIVITY OF THE EXISTING NEURONS AND POTENTIALLY REDUCE THE AMOUNT OF THE PROTEIN AGGREGATES FROM THE HUNTINGTIN PROTEIN.

CIRM HAS ONE ACTIVE CLIN1 STAGE AWARD FOR HUNTINGTON'S, WHICH IS ESSENTIALLY THE PREDECESSOR AWARD FOR THIS CURRENT APPLICATION UNDER CONSIDERATION.

THE APPLICANT HAS RECEIVED FOUR PRIOR CIRM

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AWARDS, THREE OF WHICH ARE ALL RELATED TO THE CURRENT PROJECT UNDER CONSIDERATION. THE FOURTH IS A DISCOVERY STAGE PROJECT FOR A GENE-MODIFIED VERSION OF A SIMILAR CELL PRODUCT FOR THE SAME INDICATION.

THE GRANTS WORKING GROUP RECOMMENDATION FOR THIS APPLICATION WAS A RECOMMENDATION NOT TO FUND THE APPLICATION WITH A SPLIT VOTE ACROSS 1, 2, AND 3: ONE VOTE FOR FUNDING, SIX VOTES FOR TIER II, AND EIGHT VOTES TO DO NOT FUND AND CANNOT SUBMIT FOR SIX MONTHS.

THE DEI SCORE FOR THIS APPLICATION WAS 9, AND THE CIRM TEAM RECOMMENDATION CONCURS WITH THE GRANTS WORKING GROUP RECOMMENDATION TO NOT FUND THIS APPLICATION. CHAIR IMBASCIANI.

CHAIRMAN IMBASCIANI: THANK YOU, HAYLEY. ANY QUESTIONS?

MR. FISCHER-COLBRIE: YEAH. AS WE GO THROUGH THE DISCUSSION AROUND THIS PARTICULAR APPLICATION, I WOULD LIKE TO SUGGEST A POTENTIAL AMENDMENT BECAUSE OBVIOUSLY A 3 SCORE NORMALLY ENGENDER A NO VOTE WITHIN THAT CONTEXT.

AND I'D LIKE TO PROPOSE THAT THE APPLICANT BE ALLOWED TO RESUBMIT THIS APPLICATION EARLIER THAN THE NORMAL AT LEAST SIX-MONTH TIMELINE THAT A 3

WOULD ENGENDER WITH A PERSPECTIVE OF BEING ABLE TO RESUBMIT AT THE TIME OF THE REESTABLISHMENT OF OUR FOLLOW-ON PROCESS FOR ALL OF OUR GRANTS AND REVIEWS. AND THAT IS, AGAIN, THE ONES ARE OR THE PROCESS AS PROPOSED TO BE PRESENTED TO THE BOARD IN MARCH AND EXPECTATION SOMETIME IN THE SPRING FOR REESTABLISHMENT.

SO MY PROPOSAL, THEN, IS TO ALLOW THIS APPLICATION TO BE RESUBMITTED AT THAT TIME TO BE ABLE TO PULL IN THE TIMELINE WHERE THAT MIGHT BE CONSIDERED.

CHAIRMAN IMBASCIANI: THANK YOU FOR THE MOTION. IT REQUIRES A SECOND.

VICE CHAIR BONNEVILLE: I SECOND.

CHAIRMAN IMBASCIANI: SECONDED BY MARIA. COMMENT? YSABEL, PLEASE.

MS. DURON: HAYLEY, CAN YOU EXPLAIN WHY THERE'S SUCH A DISCREPANCY BETWEEN THE SCIENTIFIC SCORE AND THE DEI SCORE? OBVIOUSLY PEOPLE CARED ABOUT THIS PROJECT AS A DEI. SO I'D LIKE TO KNOW -- IT SEEMS TO ME, QUITE FRANKLY, I'LL BE HONEST, SOMETIMES THE SERVICE AND THE INVESTIGATION IS MORE IMPORTANT THAN THE SCIENTIFIC SCORE. THAT'S THE WAY I FEEL SOMETIMES. I'M SORRY.

DR. LAM: SO I WOULD SAY OVERALL THE

REVIEW PANEL HAD VERY POSITIVE THINGS TO SAY ABOUT THE DEI ASPECTS OF THE APPLICATION, AND THEY THOUGHT THAT THE APPLICANT HAD REALLY STRONG TIES WITH THE HUNTINGTON'S DISEASE PATIENT COMMUNITY AND VERY THOUGHTFULLY PRESENTED THEIR PLAN FOR HOW THEY WERE GOING TO ACHIEVE DIVERSE ENROLLMENT AND ACCESS TO POSSIBLE PEOPLE WHO COULD RECEIVE TREATMENT.

I THINK IN TERMS OF THE SCIENTIFIC RECOMMENDATION, IT WAS FOCUSED MAINLY AROUND THE PRELIMINARY DATA THAT WAS GENERATED IN THE PRECLINICAL SETTING. AND THE OVERALL -- AS I MENTIONED, IT WAS A SORT OF A SPLIT VOTE BETWEEN A 2 AND 3, BOTH OF THOSE. AND I THINK THAT THERE WAS A GENERAL THOUGHT THAT THE PRELIMINARY DATA WASN'T AS STRONG AS THEY WOULD LIKE TO SEE AT THAT STAGE.

CHAIRMAN IMBASCIANI: OKAY. SO WE HAVE A MOTION ON THE FLOOR THAT'S BEEN SECONDED TO ALLOW THEM TO REAPPLY SHORT OF THE TYPICAL SIX-MONTH WAITING PERIOD FOR A TIER III. FURTHER DISCUSSION? YES, ANNE-MARIE.

DR. DULIEGE: I CAN MAKE A COMMENT?

CHAIRMAN IMBASCIANI: YES, I AM.

DR. DULIEGE: I UNDERSTAND SO MUCH YOUR PERSPECTIVE, BUT MAYBE YOU CAN CLARIFY FOR OTHERS WHY IN THAT CASE WE SHOULD DERAIL FROM THE

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RECOMMENDATION COMING FROM THE WORKING GROUP AND THE CIRM TEAM GIVEN HOW, AS YOU HAVE BEEN PART OF IT, VERY CAREFUL WHEN WE DO THAT. AND I'M SURE YOU'RE EXTREMELY CAREFUL.

MR. FISCHER-COLBRIE: THAT'S A GREAT QUESTION. EMBEDDED IN THERE IS A COUPLE OF FACTORS. AND ONE IS WE'VE BEEN IN A SITUATION WHERE THERE'S BEEN SOME DELAYS IN OUR PROCESS. THAT'S THE FIRST POINT.

THE SECOND THING, IF WE LOOK AT THIS CURRENT APPLICATION, IT'S RIGHT ON THE BUBBLE BETWEEN A 2 AND A 3 IN THAT ASPECT. AND THAT'S A VERY IMPORTANT CONSIDERATION.

AND THE THIRD IS, WHILE THERE IS OBVIOUSLY SOME WORK GOING ON IN HUNTINGTON'S, THIS IS OBVIOUSLY AN EXTREMELY PROBLEMATIC SET OF CONDITIONS. AND I THINK IT'S MERITORIOUS TO BE ABLE TO NOT SAY LET'S GO AHEAD WITH IT, BUT SIMPLY ALLOW THE APPLICANTS TO SUBMIT A TIMELINE THAT WOULD BE A LITTLE BIT FASTER THAN WHAT OTHERWISE WOULD OCCUR.

DR. DULIEGE: THANK YOU.

VICE CHAIR BONNEVILLE: I HAVE ANOTHER COMMENT TOO. YOU CAN IMAGINE AS WE GET CLOSER TO NEW CONCEPT PLANS; AND DEPENDING ON HOW MUCH THOSE CONCEPT PLANS CHANGE, ANYTHING THAT'S SORT OF

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EXISTING OUT THERE RIGHT NOW THAT'S SORT OF IN A REVIEW FRAME OR PROCESS MAY HAVE TO REAPPLY IN GENERAL WITH A NEW APPLICATION BECAUSE IT COULD BE DIFFERENT.

SO I THINK ACCELERATING THE PROCESS SO THAT THEY ARE ABLE TO SUBMIT AT THAT TIME MAKES SENSE JUST FROM A LOGISTICAL AND PROCESS STANDPOINT.

MR. FISCHER-COLBRIE: THANKS, MARIA. THAT'S A -- SORRY TO INTERRUPT.

CHAIRMAN IMBASCIANI: GO AHEAD.

MR. FISCHER-COLBRIE: MY APOLOGIES. THAT'S A GREAT POINT BECAUSE THE NEW PROCESS HAS THE OPPORTUNITY FOR ALL THE WAY THROUGH THE PLATFORM OF ACCELERATION, AND SO YOU DON'T WANT TO PRECLUDE THEM FROM GETTING TO THE STARTING GATE FOR THAT.

CHAIRMAN IMBASCIANI: YES.

DR. MIASKOWSKI: MARK, I JUST WANT TO CLARIFY. ARE YOU SUGGESTING THIS FOR THIS APPLICATION OR IF WE HAVE OTHERS THAT ARE IN SIMILAR CIRCUMSTANCES?

MR. FISCHER-COLBRIE: THERE'S ANOTHER ONE COMING UP THAT I WILL HIGHLY RECOMMEND THE SAME AMENDMENT TO, PARTICULARLY AROUND TIMING CONSIDERATIONS. AND, AGAIN, NONE OF THAT IS FOR AN APPROVAL. IT'S SIMPLY TO PUT IN A BUCKET THAT

ALLOWS FOR A FASTER DETERMINATION --

DR. MIASKOWSKI: I'M JUST ASKING IF WE SHOULD JUST GENERALIZE IT THEN.

MR. FISCHER-COLBRIE: I THINK IT'S BETTER JUST TO DO EACH ONE. THANKS. THANK YOU THOUGH. GOOD POINT.

CHAIRMAN IMBASCIANI: PROBABLY BECAUSE WE TAKE THESE ONE AT A TIME ANYWAY. CHRIS, THANK YOU.

OKAY. WE'RE STILL ON THE MOTION. I'D LIKE TO HEAR PUBLIC COMMENT ON THE MOTION WHICH IS AN AMENDMENT UNLESS THERE'S MORE.

MS. MANDAC: WE DO HAVE PUBLIC COMMENT FOR THIS ONE. WE'LL START IN THE ROOM FIRST WITH LESLIE. ON THE LINE WE'LL FIRST CALL ON ANNE, THEN SARAH, THEN FRANCES. YOU'LL ALL HAVE THREE MINUTES EACH. LESLIE, THE CLOCK STARTS NOW.

MS. THOMPSON: HI. I'M LESLIE THOMPSON, HUNTINGTON'S DISEASE RESEARCHER. AND I HAVE BEEN SENSE THE BEGINNING WITH CLONING OF THE GENE. I AM PI OF CLIN2 PROPOSAL 17081. THANK YOU VERY MUCH FOR THE OPPORTUNITY TO ADDRESS THE BOARD REGARDING OUR DEEP CONCERN FOR REVIEW OF OUR PROPOSAL TO ADVANCE FDA-AUTHORIZED STEM CELL-BASED THERAPY FOR HUNTINGTON'S DISEASE.

HD IS A DEVASTATING GENETIC

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NEURODEGENERATIVE DISEASE WITH NO TREATMENT THAT CHANGES THE COURSE OF THE DISEASE. CIRM HAS SUPPORTED OUR PROGRAM AND APPROVED ITS PROGRESS BEGINNING OVER 14 YEARS AGO FROM DISC TO TRAN TO CLIN. THIS WILL BE THE FIRST FDA-AUTHORIZED STEM CELL-BASED TRIAL FOR HD. THERE'S SIGNIFICANT EXCITEMENT IN THE FIELD BY FAMILIES AND RESEARCHERS FOR THE TRIAL. AND THIS TRIAL SATISFIED SPECIFIED GOALS FOR CIRM. WHY THEN ARE WE HERE TODAY?

I'D LIKE TO ADDRESS THE TWO MAIN CRITIQUES RAISED IN THE REVIEW. THE FIRST CRITIQUE WAS THAT THE CLINICAL PRECEDENT FOR FETAL TRANSPLANTS IS NOT FAVORABLE. THIS IS AN APPLES TO ORANGES COMPARISON. THE MOST RECENT FETAL TRANSPLANT TRIALS COMMENCED IN 2001, WERE COMPLETED IN 2013. BACK THEN THEY DID NOT HAVE THE ABILITY TO CONTROL SUBQUALITY AND CHARACTERIZATION AS WE DO NOW WITH A STEM CELL PRODUCT OR HAVE ACCESS TO ADVANCED SURGICAL METHODS WITH MRI GUIDANCE, UPDATED IMMUNE SUPPRESSION PROTOCOLS, AND PATIENT STAGING.

IN CONTRAST, CIRM FUNDING HAS ENABLED THE ROBUST METHODOLOGICAL ADVANCES UNDERLYING OUR FDA-AUTHORIZED TRIAL.

THE SECOND MAIN CRITIQUE WAS THAT THE PRECLINICAL DATA DO NOT SUPPORT THE RISK/BENEFIT

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RATIO AT THIS TIME. WE'VE SHOWN ROBUST IMPROVEMENT IN THREE GENETIC MOUSE MODELS INCLUDING THE MOST SEVERE. WE NOT ONLY SHOW COMPLETE PREVENTION OF A KEY MOTOR PHENOTYPE, BUT ALSO SHOWED MULTIPLE IMPROVEMENTS IN QUANTITATIVE, MOLECULAR, AND NEUROCIRCUITRY LEVEL DISEASE OUTCOMES.

BECAUSE HD IS UNIVERSALLY DEVASTATING AND FATAL, THE FDA AGREED THAT THE POTENTIAL BENEFIT GREATLY OUTWEIGHS THE RISK.

WORKING WITH CIRM THROUGHOUT THE PATH TO IT IND AND HAVING RECEIVED FDA AUTHORIZATION TO PROCEED WITH THIS CELL THERAPY, WE WERE SHOCKED TO BE TOLD THAT CELL THERAPIES FOR THIS FATAL DISORDER WERE NOT WORTH PURSUING BECAUSE OF FAILURES OF STUDIES CARRIED OUT WELL OVER A DECADE AGO.

FURTHER, THE SCORE OF A 3 FOR THIS PROPOSAL IN THE JUDGMENT OF THE FDA ARE HIGHLY CONFLICTING. WE BELIEVE THIS IS DUE TO A DISPARITY IN HOW THE ASSESSMENT OF RISK VERSUS BENEFIT TO PATIENTS WAS VIEWED. THE PRECLINICAL DATA SUPPORTING THE PROPOSED TRIAL EXCEEDS THAT FOR ANY PREVIOUS CLINICAL TRIAL OF HD. THE SIX-MONTH DELAY MANDATED BEFORE PROPOSAL SUBMISSION IN ADDITION TO DELAY FROM PAUSING CLIN APPLICATIONS IN FEBRUARY, WHICH IS WHEN WE WERE AWARDED THE FDA APPROVAL, WILL

PUSH POTENTIAL FUNDING INTO 2026 -- AND I UNDERSTAND WITH THE MOTION BEFORE THE BOARD NOW THAT THAT MIGHT CHANGE -- RESULTING IN LOSS OF CRITICAL TEAM MEMBERS AND DELAY OF POSSIBLE TREATMENT.

TODAY WE ASK THAT THIS PANEL NOT LET THE LACK OF SUCCESS IN PREVIOUS FETAL CELL HD TRIALS DEFINE THE POTENTIAL OF MODERN REGENERATIVE MEDICINE TO MOVE NEW TREATMENTS FORWARD. PLEASE CONSIDER SUPPORTING AN EXPEDITED RE-REVIEW IN RESPONSE. THANK YOU FOR YOUR TIME AND CONSIDERATION.

MS. MANDAC: THANK YOU SO MUCH, LESLIE, FOR COMING AND JOINING US. NEXT WE HAVE ANNE. YOUR THREE MINUTES STARTS NOW.

DR. ROSSER: SO I'M ANNE ROSSER. I'M A NEUROLOGIST PROFESSOR OF CLINICAL NEUROSCIENCE AT CARDIFF UNIVERSITY IN THE UK. AND SO I'M AN EXPERT IN HUNTINGTON'S DISEASE, AND I'VE ALSO SPENT THE LAST TWO AND A HALF DECADES WORKING ON CELL THERAPY SOLUTIONS FOR HUNTINGTONS'S. IMPORTANT FOR THIS APPLICATION, I'VE GOT FIRSTHAND EXPERIENCE OF FETAL CELL TRANSPLANTS IN HUMANS AS WELL AS PRECLINICAL MODELS.

SO WHAT I WANT TO DO IS REALLY TO ANSWER WHAT YOU'VE ALREADY HEARD FROM DR. THOMPSON BECAUSE A MAJOR REASON FOR REJECTING THIS APPLICATION SEEMS

TO HAVE BEEN THE NOTION THAT PREVIOUS STUDIES OF FETAL CELL TRANSPLANTS DIDN'T PRODUCE BENEFIT IN HUNTINGTON'S PATIENTS; THEREFORE, CELL THERAPY ISN'T VIABLE IN THIS DISEASE. BUT I STRONGLY BELIEVE THAT THIS CONCLUSION IS FLAWED.

FIRST AND FOREMOST, A THERAPY CANNOT BE SAID TO HAVE FAILED UNLESS IT'S BEEN GIVEN A FAIR TRIAL. AND THE FETAL TRANSPLANT STUDIES DO NOT, IN MY VIEW, REPRESENT A FAIR TRIAL OF CELL THERAPY IN HUNTINGTON'S DISEASE. THE REASON I SAY THIS IS THAT THE SUM TOTAL OF HUNTINGTON'S PATIENTS RECEIVING FETAL TRANSPLANTS ARE VERY SMALL INDEED. SO MOST STUDIES WERE ONLY IN THE REGION OF TWO TO SEVEN PATIENTS LARGELY BECAUSE COLLECTING HUMAN FETAL TISSUE IS EXTREMELY CHALLENGING.

NEVERTHELESS, SOME OF THESE SMALL STUDIES HAVE PROVIDED PROOF OF CONCEPT EVIDENCE IN THAT WHERE THERE'S GOOD EVIDENCE OF GRAFT SURVIVAL, THERE WAS ALSO GOOD EVIDENCE OF CLINICAL BENEFIT. THERE'S BEEN ONE LARGER STUDY, A BIG HD STUDY, WHICH WAS HIGHLIGHTED IN THE REVIEWERS' COMMENTS. NOW, I KNOW THIS STUDY REALLY WELL. THERE WERE A TOTAL OF 45 PATIENTS TRANSPLANTED OVER A NINE-YEAR PERIOD.

SO, FIRST, THIS IS STILL AN UNDERPOWERED STUDY. BUT THE REAL PROBLEM WITH THIS STUDY IS THAT

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MOST OF THE GRAFTS SURVIVED VERY POORLY OR NOT AT ALL. SO MOST PATIENTS IN THIS STUDY IN REALITY DIDN'T REALLY HAVE A SURVIVING GRAFT. AND, OF COURSE, WE CAN'T DRAW CONCLUSIONS ABOUT TRANSPLANT CASES WHERE THE GRAFT DID NOT SURVIVE.

SO TO SUMMARIZE, I'D SAY THAT THE FETAL TRANSPLANT WORK IN HUNTINGTON'S HAS GIVEN US SOME PROOF OF CONCEPT AND SOME ENCOURAGEMENT, BUT NO SOLID PROOF THAT FETAL CELL TRANSPLANTS EITHER DO OR DON'T WORK.

SO IMPORTANTLY, I DON'T THINK IT'S LIKELY THAT WE'RE GOING TO BE ABLE TO GET MUCH FURTHER WITH THESE FETAL CELLS WHICH MAKES IT ALL THE MORE IMPORTANT THAT STUDIES OF STEM CELL-DERIVED PRODUCT SUCH AS THIS ONE ARE ALLOWED TO MOVE FORWARD SO THAT WE CAN TEST THIS THERAPEUTIC IN HUNTINGTON'S DISEASE. AND 17081 WOULD BE THE FIRST STUDY TO TEST THE STEM CELL PRODUCT IN HUNTINGTON'S DISEASE. AND THIS IS WHY I THINK IT'S OF SUCH GREAT IMPORTANCE.

MS. MANDAC: THANK YOU, DR. ROSSER. DR. SARAH TABRIZI, YOU'RE UP NEXT TO BE FOLLOWED BY DR. FRANCES SALDANA. YOU HAVE THREE MINUTES.

DR. TABRIZI: HI. MY NAME IS SARAH TABRISI. I'M A PROFESSOR OF NEUROLOGY AT UCL IN LONDON AT UNIVERSITY COLLEGE IN LONDON. I'M ALSO

THE DIRECTOR OF THE UCL HUNTINGTON'S DISEASE CENTER, AND I'VE WORKED IN THE HUNTINGTON'S DISEASE FIELD FOR 30 YEARS. I WAS RECENTLY ELECTED AS ONE OF VERY FEW INTERNATIONAL MEMBERS OF THE U.S. NATIONAL ACADEMY OF MEDICINE IN RECOGNITION OF MY CONTRIBUTION TO HUNTINGTON'S DISEASE IN CLINICAL TRIALS.

I JUST WANT TO ADD THAT IN ADDITION TO WHAT DR. THOMPSON AND DR. ROSSER HAVE SAID IS THAT I HAVE SIGNIFICANT CONCERNS ABOUT THE PEER REVIEW PARTICULARLY RELATING TO THE COMMENTS ABOUT THE PRECLINICAL DATA THAT DR. THOMPSON PRESENTED.

DR. THOMPSON'S WORK SHOWED IN THREE GENETIC MODELS, MICE MODELS, OF HUNTINGTON'S DISEASE THAT THERE WAS SIGNIFICANT RESCUE OF THE PHENOTYPES. NOW, I'VE LED OVER 20 CLINICAL TRIALS IN HUNTINGTON'S DISEASE, AND AS I CAN REITERATE AS A PHYSICIAN, THE MOUSE MODELS ARE NOT HUMAN HUNTINGTON'S DISEASE. SO THEY CAN ONLY BE MERELY A MODEL IN WHICH TO TEST THERAPIES.

PARTICULARLY, ONE OF THE REVIEWERS COMMENTED ABOUT THE LACK OF THE RESCUE OF THE MOUSE MODELS. THE R6/2 MOUSE MODEL OF HUNTINGTON'S DISEASE WHICH THE REVIEWERS COMMENTED ABOUT IS A VERY AGGRESSIVE MOUSE MODEL. IT'S SEVERELY

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IMPAIRED, HAS DEFICITS AT BIRTH, AND DIES BY 14 WEEKS. IT'S A MODEL IN WHICH IT'S REALLY VERY DIFFICULT TO HAVE ANY THERAPEUTIC IMPACT.

DR. THOMPSON'S DATA SHOWED IN JUST OVER ONE MONTH HIGHLY SIGNIFICANT BEHAVORIAL IMPROVEMENT IN THE MICE. AND THEY ALSO SHOWED BENEFIT IN TWO OTHER SLOWER PROGRESSING MOUSE MODELS. BEYOND THAT, GIVEN THE HUNTINGTON'S AND THE NEURODEGENERATIVE DISEASE FIELD IS MOVING AWAY FROM BEHAVIOR AS A MAJOR OUTCOME IN MOUSE MODEL RESEARCH BECAUSE IT'S BEEN SO POORLY PREDICTIVE OF BENEFIT IN HUMAN STUDIES.

THEY DID ADDITIONALLY SHOW IMPROVED OUTCOMES IN ELECTRICAL ACTIVITY AND CIRCUITRY, BOTH ABSOLUTELY CRITICAL IN HUNTINGTON'S DISEASE, CONNECTIVITY BETWEEN THE TRANSPLANTED CELLS AND THE HOST CELLS, A REDUCTION OF AGGREGATION IN HOST TISSUE, PRODUCTION OF GROWTH FACTORS, CRUCIAL GROWTH FACTORS, FOR THE STRIATUM IN HUNTINGTON'S DISEASE, AND PREVENTION OF TRANSCRIPTIONAL DEFICITS.

THESE FINDINGS SHOW BROAD IMPACT OF THE CELL PRODUCT. AND I JUST WANT TO REITERATE THAT THERE ARE -- THIS IS THE MOST IMPORTANT STUDY WITH STEM CELL TRANSPLANTATION TO SEE IF REGENERATIVE MEDICINE WORKS IN HUNTINGTON'S DISEASE, A TRULY

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DEVASTATING DISORDER. AND I THINK WE NEED TO TEST THE EXPERIMENT IN HUMANS PARTICULARLY TO SEE IF THIS --

MS. MANDAC: THANK YOU VERY MUCH, DR. TABRIZI. NEXT WE HAVE DR. FRANCES SALDANA TO BE FOLLOWED BY MELODY BANDLEY. DR. SALDANA, YOU HAVE THREE MINUTES. THE FLOOR IS YOURS.

DR. SALDANA: HI. MY NAME IS FRANCES SALDANA. I'M THE CO-FOUNDER OF HD CARE, AND I'M SPEAKING FOR CLIN2-17081.

I WANT TO THANK YOU FOR SUPPORTING HUNTINGTON'S DISEASE RESEARCH IN THE PAST AND ENABLING OUR SCIENTISTS TO ACHIEVE SO MANY AMAZING BREAKTHROUGHS.

I AM NOW ASKING FOR YOUR CONTINUED SUPPORT OF CLIN2-17081. CIRM HAS GIVEN OUR HD FAMILY SO MUCH HOPE FOR A TREATMENT. I HAVE LOST ALL THREE OF MY CHILDREN AND THEIR FATHER TO HUNTINGTON'S DISEASE. AND NOW I LIVE FOR THE DAY THAT WE HAVE A TREATMENT BECAUSE MY TWO GRANDCHILDREN ARE NOW AT RISK OF INHERITING THE SAME FATAL DISEASE.

THE REALIZATION THAT WE DO NOT HAVE A TREATMENT FOR HD CONTINUES TO FRIGHTEN ME DAY IN AND DAY OUT AS MY TWO GRANDCHILDREN ARE NOW YOUNG ADULTS. THEY LIVE WITH THE UNCERTAINTY AND FEAR

THAT THEY TOO MAY SUCCUMB TO WHAT I CALL THE WORST DISEASE KNOWN TO MANKIND. THEY SAW THEIR BEAUTIFUL SIMPLY JUST WITHER AWAY UNTIL SHE WAS UNRECOGNIZABLE AND TOOK HER LAST BREATH.

MY GRANDCHILDREN NEVER GOT TO MEET THEIR GRANDFATHER AS HE DIED AT THE AGE OF 42. THEY'RE LITERALLY RUNNING SCARED AND PROBABLY WILL NOT TEST FOR THE DISEASE UNTIL THEY FEEL CONFIDENT THAT THERE MAY SOON BE A TREATMENT. I WANT THEM TO HAVE THAT HOPE. MY OWN CHILDREN, MICHAEL, MARGIE, AND MARIE, TECHNOLOGICALLY WERE ALL HD PATIENT ADVOCATES EVEN FROM THEIR WHEELCHAIR AND WHILE IN PAIN. THEY EVEN DONATED SKIN CELLS FOR HD RESEARCH, BUT THE TREATMENT WAS NOT HERE IN TIME FOR THEM.

NOT HAVING A TREATMENT SOON WILL MEAN A CONTINUED CYCLE OF HD FAMILIES AND THE RESULTANT TRAGEDY THAT WILL TAKE THE LIVES OF THOUSANDS WHO HAVE ALREADY INHERITED THE MUTANT GENE AS WELL AS FUTURE GENERATIONS WHO WILL ALSO INHERIT THE FATAL DISEASE.

I AND OTHER HD FAMILY MEMBERS PARTICIPATED RECENTLY IN THE COMMUNITY ENGAGEMENT STUDIO TO MEET WITH OTHER HD FAMILIES TO DISCUSS THE HNC TRIAL -- EXCUSE ME -- HNSC TRIAL AND WERE SO INSPIRED BY THE POSITIVE PERSPECTIVES OF THE HD

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TRIAL. THROUGH OUR 30 YEARS OF ADVOCACY, WE HAVE HAD THE OPPORTUNITY OF MEETING WITH SO MANY HD FAMILY MEMBERS, AND WE HAVE BECOME OUR OWN LARGE EXTENDED FAMILY. MANY WHO HAVE PASSED AND CHILDREN AND YOUNG ADULTS NOW, AND THAT INCLUDES MY OWN CHILDREN WHO HAD JUVENILE ONSET HUNTINGTON'S.

EVERY TIME WE LOSE ANOTHER HD FAMILY MEMBER, WE ALL SUFFER. OUR HEARTS ARE BROKEN, BUT OUR SPIRIT IS NOT, AND WE CONTINUE TO FIGHT TO SAVE OUR FAMILIES.

I WILL REMAIN COMMITTED TO SUPPORTING HD RESEARCH AND PATIENT CARE BECAUSE HOPE FOR TREATMENT IS THE ONLY THING --

MS. MANDAC: THANK YOU SO MUCH, DR. SALDANA. WE HAVE NEXT MELODY BANDLEY. MELODY, YOU HAVE THREE MINUTES.

MS. BANDLEY: HI. MY NAME IS MELODY BANDLEY, AND I'M TOO CALLING FOR THE SUPPORT OF THE APPLICATION FOR HD OR CLIN2-17081. MY HUSBAND HAS ADVANCED HUNTINGTON'S DISEASE. TWO OF MY CHILDREN HAVE ALREADY TESTED POSITIVE. MY YOUNGEST HAS NOT TESTED, BUT IS AT RISK. TIME IS OF THE ESSENCE.

THIS IS A STUDY THAT WE IN THE HUNTINGTON'S DISEASE COMMUNITY HAVE BEEN WAITING FOR

FOR YEARS. I WROTE IN THE EMAIL I SUBMITTED ABOUT HOW I'VE HAD TO WATCH MY HUSBAND CHANGE OVER THE COURSE OF TWO YEARS FROM A VERY INTELLIGENT AEROSPACE ENGINEER AND A KIND, INVOLVED FATHER TO THE POINT WHERE HE HAS DIFFICULTY COMMUNICATING AT ALL AND NEEDS FULL-TIME CARE.

I HOPE YOU'LL READ MY LETTER, AND I HOPE YOU READ THE LETTER THAT FRANCES SALDANA WROTE ABOUT THE TERRIBLE IMPACT HD HAS ON FAMILIES.

I'M NOT HERE FOR SYMPATHY. I WANT YOU TO RECONSIDER THE APPLICATION. I'M NOT A SCIENTIST, BUT RESPECTFULLY I THINK IT'S CLEAR FROM LETTERS SUBMITTED AND FROM THE INFORMATION GIVEN BY DR. ROSSER AND DR. TABRIZI THAT THE SCIENTIFIC REVIEW WAS FLAWED. I IMPLORE YOU TO PLEASE RECONSIDER YOUR DECISION.

MY DAUGHTER AND I WERE BOTH INVOLVED IN THE COMMUNITY ENGAGEMENT STUDIO FOR THE STUDY AND WERE EXCITED TO BE A PART OF IT. I BELIEVE THIS IS THE ONLY STUDY CLOSE MOVING FORWARD THAT IS EVEN ATTEMPTING TO REBUILD THE STRIATUM OR REMEDY IN ANY WAY WHAT HAS ALREADY DETERIORATED. AND I CAN'T STRESS ENOUGH. I SAW ONE OF THE COMMENTS, SOMETHING ABOUT HOW THE IMPROVEMENT WASN'T SUFFICIENT. BUT EVEN THOSE MODEST IMPROVEMENTS MAKES SUCH A

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DIFFERENCE IN DAY-TO-DAY LIVING WITH HD. AND IT SEEMS LIKE THERE'S POTENTIAL FOR MORE.

ANYWAY, HD TENDS TO WREAK HAVOC IN THE BRAIN BEFORE EVEN THE TELLTALE PHYSICAL SIGNS. I'VE SEEN THAT IN MY HUSBAND. I FEAR THAT IN MY CHILDREN. THE POSSIBILITY OF REPAIRING THAT DAMAGE TO ANY EXTENT WOULD BE REALLY BE LIFE-CHANGING. I KNOW IT'S TOO EARLY TO KNOW IF THAT ASPECT OF THE STUDY WILL BE SUCCESSFUL, BUT THE PROSPECT IS REAL. NO ONE ELSE IS EVEN CLOSE TO BRINGING IT TO THE CLINIC.

SO, AGAIN, AS I MENTIONED IN MY LETTER, I THINK THIS STUDY SEEMS PARTICULARLY IN LINE WITH CIRM'S MISSION TO FUND RESEARCH THAT IS REGENERATIVE. THE FDA HAS ALREADY GRANTED IND STATUS. IT'S SO CLOSE AND READY TO GO. PLEASE DON'T KEEP US WAITING. I'M DEEPLY APPRECIATIVE TO CIRM FOR THEIR SUPPORT OF HD RESEARCH, AND I HOPE YOU WILL PLEASE CONSIDER, PLEASE CONTINUE AND RECONSIDER THE APPLICATION FOR REGEN FOR HD. THANK YOU.

MS. MANDAC: THANK YOU VERY MUCH, MELODY. MR. CHAIR, THAT SEEMS TO BE IT FOR PUBLIC COMMENT ON THIS APPLICATION.

CHAIRMAN IMBASCIANI: THANK YOU AGAIN,

CLAUDETTE.

THE FINAL COMMENTS FROM BOARD MEMBERS. OKAY. SCOTT, I THINK MAYBE RESTATE THE MOTION AND WE CALL A VOTE.

MR. TOCHER: SURE. CHECK MY MATH PLEASE, MARK. BUT THE MOTION WILL BE TO ALLOW THE APPLICANT TO RESUBMIT THEIR APPLICATION PURSUANT TO THE REVISED CLIN PROGRAM THAT WILL BE PRESENTED FOR THE BOARD'S CONSIDERATION IN MARCH.

MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: YES.

MR. TOCHER: JUDY CHOU.

DR. CHOU: YES.

MR. TOCHER: LEONDRA CLARK-HARVEY.

DR. CLARK-HARVEY: YES.

MR. TOCHER: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MR. TOCHER: YSABEL DURON.

MS. DURON: YES.

MR. TOCHER: MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: YES.

MR. TOCHER: ELENA FLOWERS.

DR. FLOWERS: YES.

MR. TOCHER: DAVID HIGGINS.

DR. HIGGINS: YES.

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MR. TOCHER: VITO IMBASCIANI.
CHAIRMAN IMBASCIANI: YES.
MR. TOCHER: RICH LAJARA.
MR. LAJARA: YES.
MR. TOCHER: CHRIS MIASKOWSKI.
DR. MIASKOWSKI: YES.
MR. TOCHER: ADRIANA PADILLA.
DR. PADILLA: YES.
MR. TOCHER: JOE PANETTA. MARV SOUTHARD.
DR. SOUTHARD: YES.
MR. TOCHER: YAEL WYTE.
MS. WYTE: YES.
MR. TOCHER: AND KEVIN XU.
DR. XU: YES.
MR. TOCHER: THANK YOU, KEVIN. THANK YOU, MR. CHAIR. THE MOTION CARRIES.
CHAIRMAN IMBASCIANI: THANK YOU, SCOTT. HAYLEY, WE'RE GOING TO PROCEED TO OWE 83.
DR. LAM: THANK YOU. SO THE NEXT APPLICATION FOR CONSIDERATION IS CLIN2-17083. THIS IS AN APPLICATION FOR AAV GENE THERAPY TO TREAT ARRHYTHMOGENIC RIGHT VENTRICULAR CARDIOMYOPATHY.
THE APPLICANT IS REQUESTING EXACTLY 8 MILLION TO EXECUTE A PHASE 1 B CLINICAL TRIAL AND WILL PROVIDE 15.9 MILLION IN CO-FUNDING.

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A LITTLE BIT OF BACKGROUND ON THIS DISEASE. THIS TYPE OF CARDIOMYOPATHY IS AN INHERITED CONDITION WHERE THERE'S A PROGRESSIVE LOSS OF HEART MUSCLE OVER TIME. THE TYPICAL SYMPTOMS BEGIN IN THE YOUNG ADULTHOOD AND RESULTS IN A VERY HIGH RISK FOR LIFE-THREATENING HEART COMPLICATIONS THAT CAN RESULT IN PATIENTS DYING SUDDENLY.

THE CURRENT STANDARD OF CARE IS TO TREAT THE SYMPTOMS OF IRREGULAR HEART RHYTHMS WITH DRUGS OR IMPLANTABLE DEVICES. THE PROPOSED GENE THERAPY IS INTENDED TO DELIVER A FUNCTIONAL VERSION OF THE PKP2 GENE. IDEALLY THIS WOULD RESULT IN A THERAPY THAT RESTORES NORMAL FUNCTION OF THE HEART AND SLOW OR PERHAPS REVERSE DISEASE PROGRESSION.

THE CIRM PORTFOLIO HAS ONE OTHER PROJECT AT THE CLIN1 IND-ENABLING STAGE FOR THIS INDICATION. THE APPROACH IS ALSO A GENE THERAPY, BUT FOR A DIFFERENT GENE THAT INDUCES A GROWTH FACTOR THAT CIRCULATES TO THE HEART TO HELP RESTORE HEART MUSCLE FUNCTION.

THE APPLICANT'S TEAM HAS RECEIVED A PRIOR AWARD FOR A DISCOVERY STAGE GRANT FOR DEVELOPING A CANDIDATE FOR HEART FAILURE.

THE RECOMMENDATION FROM THE GRANTS WORKING GROUP WAS A UNANIMOUS RECOMMENDATION TO FUND THIS

PROJECT WITH A DEI SCORE OF 8. THE CIRM TEAM CONCURS WITH THE GRANTS WORKING GROUP RECOMMENDATION TO FUND THIS APPLICATION FOR 8 MILLION. CHAIR IMBASCIANI.

CHAIRMAN IMBASCIANI: THANK YOU, HAYLEY. I'D LIKE TO HAVE A MOTION FROM THE BOARD.

DR. SOUTHARD: SO MOVED.

CHAIRMAN IMBASCIANI: MARVIN, IT'S A MOTION TO FUND. YES. SECOND?

MR. FISCHER-COLBRIE: SECOND.

CHAIRMAN IMBASCIANI: THANK YOU, MARK. DISCUSSION TO APPLICATION FROM MEMBERS OF THE BOARD? NOW WE'RE LOOKING FOR PUBLIC COMMENT. THERE IS NO PUBLIC COMMENT.

MR. TOCHER: I DON'T SEE ANY.

CHAIRMAN IMBASCIANI: SCOTT, YOU MAY PROCEED.

MR. TOCHER: OKAY. SO THE MOTION IS TO FUND APPLICATION 17083.

MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: YES.

MR. TOCHER: JUDY CHOU.

DR. CHOU: YES.

MR. TOCHER: LEONDRA CLARK-HARVEY.

DR. CLARK-HARVEY: YES.

MR. TOCHER: ANNE-MARIE DULIEGE.
DR. DULIEGE: YES.
MR. TOCHER: MARK FISCHER-COLBRIE.
MR. FISCHER-COLBRIE: YES.
MR. TOCHER: DAVID HIGGINS.
DR. HIGGINS: YES.
MR. TOCHER: VITO IMBASCIANI.
CHAIRMAN IMBASCIANI: YES.
MR. TOCHER: RICH LAJARA.
MR. LAJARA: YES.
MR. TOCHER: ADRIANA PADILLA.
DR. PADILLA: YES.
MR. TOCHER: JOE PANETTA. MARV SOUTHARD.
DR. SOUTHARD: YES.
MR. TOCHER: YAEL WYTE.
MS. WYTE: YES.
MR. TOCHER: KEVIN XU.
DR. XU: YES.
MR. TOCHER: THANK YOU, KEVIN. THANK YOU, MR. CHAIR. THE MOTION CARRIES.
CHAIRMAN IMBASCIANI: THANK YOU, SCOTT. AND, HAYLEY, WE'RE GOING TO PROCEED TO 083.
DR. LAM: THANK YOU. SO THE NEXT APPLICATION FOR CONSIDERATION IS CLIN2-17083. THIS IS AN APPLICATION FOR AN AAV GENE THERAPY TO TREAT

ARRHYTHMOGENIC RIGHT VENTRICULAR CARDIOMYOPATHY. THE APPLICANT IS REQUESTING EXACTLY 8 MILLION TO EXECUTE A PHASE 1B CLINICAL TRIAL AND WILL PROVIDE 15.9 MILLION IN CO-FUNDING.

A LITTLE BIT OF BACKGROUND ON THIS DISEASE. THIS TYPE OF CARDIOMYOPATHY IS AN INHERITED CONDITION WITH A PROGRESSIVE LOSS OF HEART MUSCLE OVER TIME. THE TYPICAL SYMPTOMS BEGIN IN THE YOUNG ADULTHOOD AND RESULTS IN A VERY HIGH RISK FOR LIFE-THREATENING HEART COMPLICATIONS THAT CAN RESULT IN PATIENTS DYING SUDDENLY.

THE CURRENT STANDARD OF CARE IS TO TREAT THE SYMPTOMS OF IRREGULAR RHYTHMS WITH DRUGS OR IMPLANTABLE DEVICES.

THE PROPOSED GENE THERAPY IS INTENDED TO DELIVER A FUNCTIONAL VERSION OF THE PKP2 GENE. IDEALLY THIS WOULD RESULT IN A THERAPY THAT RESTORES NORMAL FUNCTION OF THE HEART AND SLOW OR PERHAPS REVERSE DISEASE PROGRESSION.

THE CIRM PORTFOLIO HAS ONE OTHER ACTIVE PROJECT AT THE CLIN1 IND-ENABLING STAGE FOR THIS INDICATION. THE APPROACH IS ALSO A GENE THERAPY BUT FOR A DIFFERENT GENE THAT INDUCES A GROWTH FACTOR THAT CIRCULATES TO THE HEART TO HELP RESTORE HEART MUSCLE FUNCTION.

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THE APPLICANT TEAM HAS RECEIVED A PRIOR AWARD FOR A DISCOVERY STAGE GRANT FOR DEVELOPING A CANDIDATE FOR HEART FAILURE.

THE RECOMMENDATION FROM THE GRANTS WORKING GROUP WAS A UNANIMOUS RECOMMENDATION TO FUND THIS PROJECT WITH A DEI SCORE OF 8. THE CIRM CONCURS WITH THE GRANT WORKING GROUP RECOMMENDATION TO FUND THIS APPLICATION FOR 8 MILLION.

CHAIR IMBASCIANI.

CHAIRMAN IMBASCIANI: THANK YOU, HAYLEY. I'D LIKE TO HAVE A MOTION FROM THE BOARD.

DR. SOUTHARD: SO MOVED.

CHAIRMAN IMBASCIANI: MARVIN, IT'S A MOTION TO FUND? YES. SECOND?

MR. FISCHER-COLBRIE: SECOND.

CHAIRMAN IMBASCIANI: THANK YOU, MARK. DISCUSSION ON THIS APPLICATION FROM MEMBERS OF THE BOARD. HOW ARE WE LOOKING FOR PUBLIC COMMENT? THERE IS NO PUBLIC.

MR. TOCHER: I DON'T SEE ANY.

CHAIRMAN IMBASCIANI: SCOTT, YOU MAY PROCEED.

MR. TOCHER: SO THE MOTION IS TO FUND APPLICATION 17083.

MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: YES.
MR. TOCHER: JUDY CHOU.
DR. CHOU: YES.
MR. TOCHER: LEONDRA CLARK-HARVEY.
DR. CLARK-HARVEY: YES.
MR. TOCHER: ANNE-MARIE DULIEGE.
DR. DULIEGE: YES.
MR. TOCHER: MARK FISCHER-COLBRIE.
MR. FISCHER-COLBRIE: YES.
MR. TOCHER: DAVID HIGGINS.
DR. HIGGINS: YES.
MR. TOCHER: VITO IMBASCIANI.
CHAIRMAN IMBASCIANI: YES.
MR. TOCHER: RICH LAJARA.
MR. LAJARA: YES.
MR. TOCHER: ADRIANA PADILLA.
DR. PADILLA: YES.
MR. TOCHER: JOE PANETTA. MARV SOUTHARD.
DR. SOUTHARD: YES.
MR. TOCHER: YAEL WYTE.
MS. WYTE: YES.
MR. TOCHER: KEVIN XU.
DR. XU: YES.
TR. TOCHER: GREAT. THANK YOU. MOTION CARRIES.

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CHAIRMAN IMBASCIANI: THANK YOU. AND, HAYLEY, FOR THE FINAL ONE.

DR. LAM: ALL RIGHT. FINAL APPLICATION, CLIN2-17091. THIS IS A GENE THERAPY FOR SPASTIC PARAPLEGIA TYPE 50. THE APPLICANT IS REQUESTING 14.9 MILLION IN FUNDING WHILE PROVIDING JUST UNDER 10 MILLION IN CO-FUNDING TO COMPLETE A PHASE 3 TRIAL.

SOME BACKGROUND ON THIS PROJECT. SPG50 IS A RARE NEURODEGENERATIVE DISEASE CAUSED BY A MUTATION IN THE ADAPTER PROTEIN COMPLEX 4 GENE, WHICH IS INVOLVED IN PROTEIN TRAFFICKING IN CELLS. THE SYMPTOMS BEGIN EARLY IN LIFE AND START AS MUSCLE WEAKNESS, BUT PROGRESS OVER TIME TO WORSENING STIFFNESS, EPILEPSY, EVENTUALLY PARALYSIS.

THERE ARE NO APPROVED TREATMENTS FOR THIS DISEASE, AND THE CURRENT STANDARD OF CARE TREATS THE SYMPTOMS ONLY.

THE PROPOSED GENE THERAPY WILL PROVIDE A FUNCTIONAL COPY OF THE DEFECTIVE GENE, IDEALLY SLOWING OR REVERSING DISEASE PROGRESSION.

CIRM CURRENTLY HAS NO TRANSLATIONAL OR CLINICAL AWARDS ADDRESSING THIS INDICATION. AND THE APPLICANT HAS AN ACTIVE CLINICAL CLIN1 IND-ENABLING STAGE AWARD FOR A DIFFERENT NEURODEVELOPMENTAL

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DISEASE USING A SIMILAR GENE THERAPY APPROACH.

THE RECOMMENDATION FROM THE GRANTS WORKING GROUP WAS A UNANIMOUS VOTE TO NOT FUND THIS APPLICATION WITH A DEI SCORE OF 8. THE CIRM TEAM CONCURS WITH THE GRANTS WORKING GROUP RECOMMENDATION TO NOT FUND THIS APPLICATION. CHAIR IMBASCIANI.

CHAIRMAN IMBASCIANI: THANK YOU, HAYLEY. I'LL RECOGNIZE MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: YEAH. SIMILAR TO THE LAST APPLICATION THAT HAD A 3 WITH RESPECT TO TIME IS OF THE ESSENCE ON THIS CONDITION, I WOULD LIKE TO MAKE THE PROPOSAL AND RECOMMENDATION TO ALLOW THE APPLICANT TO RESUBMIT A PROPOSAL AT THE TIME THAT THE NEW PROCESSES ARE IMPLEMENTED TO BE ABLE TO PULL IN THE TIME CONSIDERATION. AND, AGAIN, NOT FROM THE PERSPECTIVE OF VOTING OR CHANGING OTHER PEOPLE'S OPINIONS OF THE PROCESS, BUT RATHER FROM THE TIME PERSPECTIVE, IN PARTICULAR WITHIN THE CONTEXT OF I BELIEVE THERE'S NEW DATA AND THERE'S ALSO CIRCUMSTANCES WHERE I UNDERSTAND PATIENTS ARE POTENTIALLY LINED UP TO BE ABLE TO PARTICIPATE IN THIS TRIAL. SO TIME IS A VERY IMPORTANT FACTOR HERE.

CHAIRMAN IMBASCIANI: THANK YOU, MARK. THAT SOUNDED EASIER THE SECOND TIME.

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MAY I HAVE A SECOND PLEASE? MARV SECONDED? SIMILAR TO THE LAST TWO APPLICATIONS AGO, CONVERSATION, DISCUSSION FROM BOARD MEMBERS ON THIS APPLICATION? OKAY. I'LL OPEN IT UP TO COMMENT FROM THE MEMBERS OF THE PUBLIC, APPLICATION 17091.

MS. MANDAC: WE DO HAVE MEMBERS OF THE PUBLIC IN PERSON AS WELL AS ONLINE. WE'LL START WITH THE MEMBERS IN PERSON, WHICH ARE TERRY AND EMMA. IF YOU COULD PLEASE COME UP TO THE MICROPHONE. YOU'LL HAVE THREE MINUTES. TIME STARTS NOW.

MS. PIROVOLAKIS: GOOD AFTERNOON. MY NAME IS EMMA PIROVOLAKIS AND I'M HERE TODAY AS A MOTHER, DESPERATE, HOPEFUL, AND DETERMINED. MY HUSBAND WAS BORN IN SAN DIEGO, BUT I'M HERE FOR SCHOOL FOR THE GROWTH THAT THIS STATE OFFERS. CALIFORNIA HAS ALWAYS BEEN A LEADER IN MEDICAL ADVANCEMENTS AND A BEACON OF HOPE FOR FAMILIES LIKE MINE.

THIS STATE HAS SET THE STANDARD FOR PUSHING BOUNDARIES IN HEALTHCARE AND CHANGING LIVES. MY SON, WHO MOST OF YOU MET THIS MORNING, HAS SPG50, A RARE AND DEVASTATING DISEASE. SPG50 IS PROGRESSIVE. IT TAKES AWAY A CHILD'S ABILITY TO WORK, TO TALK, TO DO ANYTHING INDEPENDENTLY. TIME IS NOT ON OUR SIDE. AT THREE YEARS OLD HE IS PURE

JOY, BUT THE REALITY IS, WITHOUT TREATMENT, HE WILL BECOME A HUSK OF THE PERSON HE IS NOW.

HE WILL BECOME PARALYZED, REDUCED TO A VEGETATIVE STATE. AND AS HIS PARENTS, WE WILL BE DOOMED TO WATCH HIM SLIP AWAY. HERE'S THE CRUEL REALITY. A TREATMENT ALREADY EXISTS. A GENE THERAPY DESIGNED TO ADDRESS SPG50 HAS BEEN DEVELOPED. IT IS THERE IN A FREEZER READY, BUT IT IS INACCESSIBLE, NOT BECAUSE IT DOESN'T WORK, NOT BECAUSE IT'S UNSAFE, BUT BECAUSE THE CLINICAL TRIALS LACK THE FUNDING TO MOVE FORWARD TO REACH CHILDREN LIKE MY SON. THE INJUSTICE OF THAT IS UNBEARABLE.

MY CHILD'S FUTURE IS IN PERIL, NOT BECAUSE THERE ISN'T A SOLUTION, BUT BECAUSE WE CAN'T AFFORD TO DELIVER TO HIM. IMAGINE IT WERE YOUR CHILD AND YOU'RE STANDING IN FRONT OF A LOCKED DOOR. ON THE OTHER ON SIDE IS HOPE, A CHANCE FOR THEM TO LIVE A BETTER LIFE, A LIFE NOT DEFINED BY CRUEL DISEASE, BUT YOU CAN'T OPEN THAT DOOR BECAUSE THERE ISN'T ENOUGH MONEY TO TURN THE KEY.

THAT'S WHERE WE ARE AND THAT'S WHERE FAMILIES LIKE MINE ARE EVERY DAY. AS WE SPEAK, MY SON'S CONDITION WORSENS. SPG50 WON'T PAUSE. IT DOESN'T GIVE US THE LUXURY OF WAITING. IF THESE TRIALS DON'T HAPPEN NOW, IT WILL BE TOO LATE FOR MY

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SON AND FOR SO MANY OTHERS.

EACH DAY WITHOUT ACTION MEANS IRREVERSIBLE DAMAGE. WHAT'S EVEN MORE DEVASTATING IS KNOWING THAT WITH YOUR HELP, WITH THIS GRANT, WE COULD MOVE FORWARD. WE COULD GIVE THESE CHILDREN A CHANCE. THIS IS NOT ABOUT FUNDING RESEARCH. THIS IS ABOUT SAVING LIVES. THIS IS ABOUT JUSTICE, ABOUT GIVING OUR SON THE SAME CHANCE ANY CHILD DESERVES. WE ARE NOT ASKING FOR A MIRACLE. WE ARE ASKING FOR THE MEANS TO DELIVER A MIRACLE, A DRUG THAT'S ALREADY BEEN CREATED. PLEASE DON'T LET TIME RUN OUT. A YEAR HAS ALREADY PASSED WITHOUT HIM RECEIVING THE TREATMENT HE NEEDS. APPROVING THIS GRANT COULD MEAN EVERYTHING. THE DIFFERENCE BETWEEN A LIFE LOST AND A LIFE LIVED, YOU HAVE THE POWER TO UNLOCK THAT DOOR. I AM BEGGING YOU AS A MOTHER ONTO HELP US OPEN IT. THANK YOU.

MS. MANDAC: THANK SO MUCH, EMMA. WE'RE ACTUALLY MOVING TO THE ZOOM FIRST. IF WE COULD GO TO REBECCA LOCKARD. WE HAVE THREE MINUTES. YOUR CLOCK STARTS NOW. REBECCA, CAN YOU PLEASE UNMUTE? REBECCA, CAN YOU HEAR US?

MS. LOCKARD: I APOLOGIZE. YES.

MS. MANDAC: YOUR CLOCK STARTS NOW.

MS. LOCKARD: THANK YOU. HELLO, MEMBERS

OF THE BOARD. MY NAME IS REBECCA AND I'M MOM TO NAOMI AND JACK. MY WORLD COLLAPSED ON MAY 12, 2023, WHEN I FOUND OUT THAT MY DAUGHTER WAS DIAGNOSED WITH SPG50 AND THAT THERE WAS A 25-PERCENT CHANCE THAT MY UNBORN SON WOULD ALSO BE AFFECTED.

WHEN OUR FEARS CAME TRUE FIVE WEEKS AFTER JACK'S BIRTH WHEN HIS GENETIC TEST SHOWED THAT HE WAS POSITIVE TOO. I LEARNED THAT MY CHILDREN WOULD SLOWLY DETERIORATE, BECOMING PARAPLEGIC IN ELEMENTARY SCHOOL, QUADRIPLEGIC IN HIGH SCHOOL, AND PERHAPS FACE AN EARLY DEATH. THE IDEA THAT THEY WOULD BE NONVERBAL AND HAVE SEVERE COGNITIVE IMPAIRMENTS WAS DEVASTATING.

TWO DAYS AFTER NAOMI'S DIAGNOSIS, I SOBBED ON MOTHER'S DAY WHEN THE SHOCK LIFTED A BIT AND I REALIZED THAT I WOULD NEVER HEAR MY KIDS SAY MAMA.

THE ONLY THING THAT GOT ME THROUGH THOSE DARK DAYS WAS THE HOPE OF A GENE THERAPY DRUG THAT WAS ALREADY DEVELOPED AND BEING TESTED ON AFFECTED KIDS. THE IDEA THAT MY CHILDREN'S FACE SHOWED THEIR DEGENERATION WAS NOT SET IN STONE CONTINUES TO HELP ME FIGHT FOR EACH DAY.

JACK WAS TREATED IN DECEMBER 2023, A FEW DAYS SHY OF HIS SIX-MONTH BIRTHDAY. HE WAS THE YOUNGEST CHILD TO EVER RECEIVE ANY TYPE OF

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INTRATHECAL GENE THERAPY. HE FACED NO ADVERSE REACTIONS, AND HE HAS THRIVED SINCE RECEIVING THE DRUG.

SO, MEMBERS OF THE BOARD, IF YOU WANT TO KNOW IF THE DRUG WORKS, LOOK AT MY CHILDREN. THEY HAVE THE SAME PARENTS, THE SAME THERAPY PROVIDERS, THE SAME DOCTORS, THE EXACT SAME MUTATED GENES, AND YET DIFFERENT OUTCOMES.

JACK IS TWO YEARS YOUNGER THAN NAOMI AND SURPASSED HER DEVELOPMENTALLY. THE ONLY EXPLANATION FOR THE DIFFERENCE IN DEVELOPMENT IS THIS TREATMENT. TODAY MY DAUGHTER NAOMI IS A HAPPY, BRIGHT, AND CLEVER THREE AND A HALF YEAR OLD LITTLE GIRL. SHE DID LEARN TO SAY MAMA AND DADA AND BABA FOR BROTHER TOO. SHE CRAWLS AROUND THE HOUSE AND HAS TAKEN A FEW STEPS IN HER GAIT TRAINER. SHE HAS VERY POOR FINGER STRENGTH, BUT SHE'S FIGURED OUT HOW TO BITE HER STUFFIES TO ACTIVATE THE SOUND AND USING BUTTONS HIDDEN WITHIN THEM. SHE WILL LOSE ALL OF THIS AND SO MUCH MORE WITHOUT THE TREATMENT THAT EXISTS AND IS JUST NOT ACCESSIBLE TO HER.

YOUR FUNDING WILL GIVE HOPE TO THE FAMILIES AND FRIENDS OF KIDS WITH SPG50 WHO WORRY DAILY ABOUT WHAT THEIR FUTURE HOLDS. IT WILL HELP SHOW THAT RARE DISEASES ARE WORTH DEVELOPING

TREATMENTS FOR, THAT THEY ARE VIABLE TO MOVE THROUGH THE PHASES OF A CLINICAL TRIAL, AND INTO THE PUBLIC. IT WILL GIVE VOICE TO THE SMALLEST COMMUNITIES WHO SUFFER ALONE BECAUSE THEIR CONDITION ONLY AFFECTS A TINY PORTION OF THE POPULATION. AND MOST IMPORTANTLY, IT WILL GIVE CHILDREN A CHANCE AT A FULL AND FRUITFUL LIFE.

I WANT YOU TO RECONSIDER YOUR DECISION. THIS PROGRAM IS THE BEST CHANCE THAT KIDS WITH SPG50 HAVE AT TREATING THEIR CONDITION. IT IS THE ONLY TREATMENT THAT WILL EXIST FOR THEM AT AN AGE WHERE IT WILL MAKE A DIFFERENCE, A CHANCE TO GIVE THEM A HAPPY AND POSITIVE LIFE, NOT ONE FILLED WITH PAIN AND SUFFERING. I'M ASKING FOR YOU TO HELP ME ACHIEVE THAT GOAL. THANK YOU.

MS. MANDAC: THANK YOU VERY MUCH, REBECCA. NEXT WE HAVE DR. DARIUS. IF YOU COULD PLEASE UNMUTE. DR. DARIUS, IF YOU COULD PLEASE UNMUTE. OKAY. WE'LL MOVE RIGHT ON TO THE NEXT ONE. DR. GOTLIEB, IF YOU COULD PLEASE UNMUTE TO BE FOLLOWED BY DR. GRAY. DR. GOTLIEB, YOU HAVE THREE MINUTES.

DR. GOTTLIEB: YES. I'M UNMUTED. SO MY NAME IS DR. KEITH GOTLIEB. I'M THE PI FOR THIS ONE, FOR CLIN2-17091. I'M HONORED TO SPEAK TO YOU, TO

THE BOARD, TODAY NOT SO MUCH OUT OF FRUSTRATION, BUT ABOUT A DEEP CONVICTION AND UNWAVERING HOPE FOR THIS PROGRAM. IT'S AN EXTRAORDINARY OPPORTUNITY TO CHANGE LIVES AS YOU JUST HEARD FROM TWO INDIVIDUALS AND FAMILIES.

I HAVE DEEP ROOTS IN CALIFORNIA. IN FACT, I'VE BEEN HERE SINCE 1996. MY PH.D. IS ACTUALLY FROM THE UNIVERSITY OF CALIFORNIA IRVINE. MY KIDS WERE BORN HERE. I OWN A HOUSE HERE. MY WIFE HAS A COMPANY HERE. AND MY ENTIRE CAREER HAS BEEN IN COMPANIES IN CALIFORNIA. I'VE PAID TAXES HERE FOR 30 YEARS, INCLUDING VOTING FOR THE PROPOSITIONS THAT SUPPORT CIRM IN 2004 AND 2020. AND I WAS HOPING, HOPING WITH ALL MY MIGHT THAT THIS PROGRAM WOULD BE SUPPORTED BY ALL THE WORK THAT HAS GONE INTO THE APPLICATION AND THE AMAZING TECHNOLOGY THAT THIS COULD BE FOR THESE KIDS.

SPG50 WAS SELECTED BY THE VERY PRESTIGIOUS JUST BESPOKE THERAPY CONSORTIUM WHICH WAS LED BY THE FOUNDATIONS FOR THE NATIONAL INSTITUTE OF HEALTH. THIS WAS SUPPOSED TO BE A COLLABORATION, IS A COLLABORATION, IN FACT, WITH NIH, THE FDA, AND CIRM ITSELF, SIGNIFYING A PROMISED SCIENTIFIC RIGOR OF THIS PROGRAM. AND THIS RECOGNIZES THE URGENCY AND IMPORTANCE OF ADVANCING IMMUNE THERAPIES FOR RARE

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DISEASE.

YET WHEN I LOOKED AT THE COMMITTEE'S FEEDBACK, I WAS COMPLETELY BLOWN AWAY BY WHAT I SAW. THE CRITIQUES WERE A LACK OF UNDERSTANDING, A MISUNDERSTANDING OF THE UNIQUE CHALLENGES OF ULTRA-RARE CONDITIONS. ONE REVIEWER EVEN SAID THAT OUTSIDE OF A VAGUE STATEMENT ABOUT POTENTIAL IMPROVEMENTS THAT DID NOT PROVIDE ENOUGH INFORMATION ABOUT EFFICACY, AND WE HAD ALREADY TREATED SIX CHILDREN. YOU JUST HEARD ABOUT ONE OF THEM. AND WE ALREADY PRESENTED THIS INFORMATION IN OUR PACKAGE.

ANOTHER ONE SAYS THAT THE IMPACT COULD BE MODEST AT BEST. BUT ANY CHANGE IN THESE CHILDREN, MUCH LIKE THE PREVIOUS ONES, A SINGLE STEP TAKEN, A WORD SPOKEN, A MOMENT OF RECOGNITION TO THEIR PARENTS CAN BE LIFE-CHANGING.

THIS WAS LIKE A COMPLETE REALITY THAT THESE FAMILIES ARE FACING AT ALL TIMES. I EMPOWER THE COMMITTEE TO EMBRACE THE RESPONSIBILITY THAT YOU HAVE TO FUND THIS SCIENCE. AND I WANT TO END WITH A QUOTE FROM DR. PETER MARKS, WHO'S CURRENTLY THE DIRECTOR OF CBER AT THE FDA WHO STATED THAT RARE DISEASE RESEARCH SERVES AS A TESTING GROUND FOR ADVANCEMENTS THAT WILL ULTIMATELY BENEFIT BROADER PATIENT POPULATIONS. THAT'S WHAT SPG LAYS THE

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FOUNDATION FOR FOR THIS POPULATION. I ENCOURAGE YOU --

MS. MANDAC: THANK YOU VERY MUCH, DR. GOTLIEB. NEXT WE HAVE DR. GRAY TO BE FOLLOWED BY DR. KAKKIS. DR. GRAY, IF YOU COULD PLEASE UNMUTE. DR. GRAY, IF YOU COULD PLEASE UNMUTE. ALL RIGHT. WE'LL MOVE ON THEN TO DR. KAKKIS. DR. KAKKIS, IF YOU COULD PLEASE UNMUTE.

DR. KAKKIS: HELLO, CIRM BOARD. PLEASED TO BE ABLE TO TALK TO YOU TODAY ABOUT THIS APPLICATION. I'M A MEDICAL GENETICIST M.D./PH.D. TRAINED HERE IN CALIFORNIA, AND I'VE BEEN WORKING IN BIOTECHNOLOGY SINCE LEAVING UCLA AT BIOMARIN AND ULTRAGENICSS. I HAVE PERSONALLY BEEN INVOLVED IN 11 DRUG APPROVALS AND HAVE A RECORD OF 11 OUT OF 13 PROGRAMS ENTER THE CLINIC. SO I HAVE A DEEP EXPERIENCE AND UNDERSTANDING HOW TO PICK GENE THERAPY PROGRAMS OR OTHER TYPES OF PROGRAMS THAT CAN SUCCEED AND MOVE FORWARD TO SUCCESSFUL PRODUCTS.

I GOT INVOLVED WITH THIS PROGRAM EARLY ON. AND PERSONALLY, BECAUSE OF ITS POTENTIAL FOR SUCCESS, DONATED $800,000 IN THE EARLY DAYS TO GET ENOUGH MANUFACTURING DONE FOR MR. PIROVOLAKIS TO TREAT HIS SON. AND SO I'M DEEPLY KNOWLEDGEABLE WITH THIS, AND OUR TEAM HERE HAS HELPED TERRY AT VARIOUS

POINTS IN TIME. UNFORTUNATELY, ULTRAGENICS ITSELF, MY COMPANY, CANNOT DEVELOP THIS TREATMENT BECAUSE IT'S JUST TOO RARE A DISEASE FOR US. WE DO A LOT OF RARE DISEASE, BUT THIS ONE IS JUST TOO SMALL. BUT I HAVE HIGH FAITH IN IT, AND I BELIEVE THE REVIEW THAT I READ WAS COMPLETELY OFF BASE. AND I HAVE NO UNDERSTANDING OF HOW THEY GOT UP TO THE CONCLUSIONS THEY HAD. I REALLY THINK THIS NEEDS TO BE REDONE.

IF YOU LOOK AT WHAT'S GOING ON RIGHT NOW IN AV9 GENE THERAPY AND SMA, BOTH I.V. AND I.T., IT HAS BEEN A POWERFUL, DRAMATIC CHANGE IN OUTCOME FOR THOSE PATIENTS. SPG50 IS VERY MUCH LIKE SMA IN TERMS OF THE TARGET NEURONS AND THE TECHNOLOGY BEING USED. THE PROBABILITY OF SUCCESS IS HIGH AND THEY HAVE SUCCESSFULLY ALREADY TREATED SIX KIDS, INCLUDING MR. PIROVOLAKIS' OWN SON.

THE TRUTH IS THAT THIS IS A PROGRAM THAT'S HIGHLY LIKELY TO BENEFIT PATIENTS. AND HOW PERFECTLY IT BENEFITS THE SPINAL CORD MAYBE IN QUESTION, BUT THE TREATMENT SO FAR HAS SHOWN BENEFIT. AND I WOULD SAY FOR MANY OF THE COMPLEX THINGS THAT CIRM HAS TO FUND AND DO, THIS IS ONE OF THOSE PROGRAMS THAT WILL BE A WIN AND IMPORTANT PLACE FOR CIRM TO CONTRIBUTE TO ACTUAL POSITIVE MOVEMENT AND WITH A DRUG THAT ACTUALLY WORKS AND

GETS TO PATIENTS.

I THINK THAT THE REVIEW WAS OFF BASE. I'M NOT SURE WHY IT WAS, WHERE THE POINTS WERE. BUT I THINK FROM WHAT I KNOW AS A RARE DISEASE DRUG DEVELOPER WITH A HIGH RECORD OF SUCCESS IN THESE PROGRAMS, HIGH VALUE, HIGH SUCCESS, AND IT'S MOVED TO THIS VERY CRITICAL PLACE TO TREAT MANY KIDS WHO WILL HAVE NO OTHER SHOT. AND I WISH I COULD DO IT AT ULTRAGENICS AND TERRY HAS ASKED ME MULTIPLE TIMES, BUT I CANNOT. BUT I'VE DONE EVERYTHING I CAN TO HELP THIS GET DONE. I THINK CIRM IS ONE OF THE THOSE PLACES THAT CAN HELP THESE DREAMS COME TRUE, AND I THINK THE SCIENCE SHOULD BE REEVALUATED. I DO THINK IT'S A LOT MORE SOLID THAN EVERYTHING WE HAVE, AND THE SCIENCE AROUND SMA AND AV9 TELLS US THIS IS A GOOD PATH FORWARD FOR SPG50, AND I THINK THE DATA TO DATE SUPPORTS THAT. I WANT TO GIVE MY WHOLEHEARTED RECOMMENDATION TO YOU. REDO THE SCIENCE REVIEW--

MS. MANDAC: THANK YOU VERY MUCH, DR. KAKKIS. TERRY PIROVOLAKIS, YOU HAVE THREE MINUTES.

MR. PIROVOLAKIS: I COME TO YOU TODAY AS A FATHER OF A RARE DISEASE CHILD, A STRONG CIRM PARTNER, A BIOTECH CEO, AND MOST IMPORTANTLY THE VOICE FOR THE RARE DISEASE COMMUNITY. I AM HERE TO

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FIGHT FOR THE LIVES OF CHILDREN, CHILDREN WHO OFTEN WILL NOT SURVIVE WITHOUT THE SUPPORT AND LEADERSHIP OF ORGANIZATIONS LIKE CIRM.

IN 2022 I PLEADED TO THE BGDC BOARD TO TAKE ON SPG50 DESPITE DESPERATE TO SAVE MORE CHILDREN. THAT PLEA LED TO THE CREATION OF ALTHENA THERAPEUTICS, A CALIFORNIA COMPANY AND AN HISTORIC COLLABORATION INVOLVING CIRM, THE BGDC, NIH, FNIH, AND CEDARS-SINAI. TOGETHER WE SOUGHT TO DELIVER HOPE WHERE THERE WAS NONE. WHAT WE DIDN'T ANTICIPATE WAS THAT COLLABORATION WOULD REQUIRE US TO APPLY TO CIRM, RAISE MILLIONS IN CO-FUNDING, AND NOW STAND BEFORE YOU TO APPEAL A DECISION THAT THOROUGHLY DISTINGUISHED THAT HOPE, NOT JUST FOR SPG50, BUT EVERY CHILD THAT IS AFFECTED BY RARE DISEASE.

OUR DRUG IS READY. OUR CHILDREN ARE DESPERATELY WAITING. THE FOREMOST LEADERS IN THE FIELD ARE FULLY COMMITTED TO THIS EFFORT, AND WITHIN MONTHS WE COULD BEGIN SAVING THESE CHILDREN. WE HAVE A CLEAR PATH TO ADVANCE THIS PROGRAM TOWARDS APPROVAL.

KEITH HAS ALREADY OUTLINED THE TECHNICAL FLAWS IN THE REVIEW PROCESS. THE REVIEWERS' COMMENTS ARE NOT JUST MISINFORMED, BUT THEY ARE

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DANGEROUS. TO SUGGEST THAT A THERAPY COULD WORSEN OUTCOMES OR PRECLUDE FUTURE TREATMENT OPTIONS IGNORES THE SCIENCE, DISMISSES THE URGENCY, UNDERMINES THE MISSION WE ALL SHARE.

THE CHILDREN -- THESE CHILDREN HAVE NO TIME TO WAIT FOR ANOTHER THERAPY THAT WILL NEVER COME. BY VOTING AGAINST OUR PROGRAM, THIS BOARD WOULD SEND A CHILLING MESSAGE TO THE RARE DISEASE COMMUNITY THAT CIRM DOES NOT STAND BY ITS COMMITMENTS TO THE BGTC, NIH, OR FNIH THAT IT'S NOT SERIOUS ABOUT RARE DISEASE, THAT DOES NOT BELIEVE IN THE PROMISE OF BRAIN-DIRECTED GENE THERAPIES. IT DOES NOT RECOGNIZE THE VALUE OF ADAPTIVE TRIAL DESIGNS, APPROACHES THE FDA IS ALREADY EMBRACING TO ACCELERATE TREATMENTS FOR PATIENTS IN URGENT NEED. WE CANNOT LET THIS HAPPEN. I AM BEGGING YOU TO VOTE TO APPROVE OUR PROGRAM. THIS IS ABOUT MORE THAN FUNDING. IT'S ABOUT SAVING LIVES.

IT'S ABOUT SHOWING THE RARE DISEASE COMMUNITY THAT CIRM STANDS WITH THEM, THAT IT BELIEVES IN BOLD, GROUNDBREAKING SCIENCE, AND THAT IT'S WILLING TO FIGHT FOR CHILDREN WITHOUT OTHER OPTIONS.

IF A LEADING PROGRAM LIKE OURS WITH A NATURAL HISTORY SETTING IN CHILDREN'S HOSPITAL,

PARTNERED BY THE BESPOKE GENE THERAPY CONSORTIUM, NIH, AN NINDS, HAS SUPPORT FROM BIOTECHS LIKE ULTRAGENICS, ROCHE, CHARLES RIVER, AND ENVIROGEN, HAS TREATED SIX CHILDREN, HAS FDA APPROVAL FOR A PHASE 3 DOES NOT GET FUNDING, WHAT PROGRAM WILL? THE VOTE IS NOT JUST A DECISION. IT'S A DECLARATION THAT CIRM'S COMMITMENT TO RARE DISEASE AND THE FUTURE OF MEDICINE. PLEASE DO THE RIGHT THING. TAKE ACCOUNTABILITY, APPROVE THIS PROGRAM, AND LET'S SAVE THESE CHILDREN TOGETHER. THANK YOU.

MS. MANDAC: THANK YOU VERY MUCH. LAST CALL FOR PUBLIC COMMENT ON THIS APPLICATION, 17091. MR. CHAIR, THERE ARE NO MORE HANDS RAISED.

CHAIRMAN IMBASCIANI: THANK YOU, CLAUDETTE, FOR MANAGING THAT. WE HAVE A MOTION ON THE FLOOR. ARE THERE ANY FINAL COMMENTS FROM BOARD MEMBERS ON BOARD MEMBER FISCHER-COLBRIE'S MOTION? J.T.

DR. THOMAS: SO I'D LIKE TO ADDRESS A COUPLE OF COMMENTS TO BOTH THE LAST -- NOT THE LAST -- THIS AND THE HUNTINGTON'S APPLICATION AS WELL.

WE HAVE A PROCESS HERE, AND THE PROCESS HAS FOR 20 YEARS BEEN FOUNDED IN HAVING ROBUST PEER REVIEW OF PROPOSALS THAT COME IN FOR FUNDING. IF WE

DEVIATE FROM THAT, WE WILL BE IN A POSITION WHERE WE'LL BE CHANGING THE WAY THINGS HAVE ALWAYS BEEN. HERETOFORE HAVING GRANTS THAT WERE SCORED TIER III MEANT INEXORABLY THAT THEY WOULD NOT BE ABLE TO REAPPLY UNTIL A MINIMUM OF SIX MONTHS, WHICH IN THE CASE OF THESE TWO PROPOSALS WOULD PUT THEM OUT INTO THE SUMMER.

WE HAD A TREMENDOUS AMOUNT OF INPUT FROM FAMILY MEMBERS, MEMBERS OF THE PUBLIC, VARIOUS OTHERS ON BOTH OF THESE APPLICATIONS. AND WE HEARD THAT. AND IN SO DOING, BECAUSE WE CAN'T SIT HERE AS A BOARD AND EVALUATE SCIENTIFIC CRITIQUES OF OUR REVIEW PROCESS IN ANY WAY THAT WOULD BE COMPETENT. SO THE BEST WE CAN TO IS TO TRY TO GET THEM RECONSIDERED AS SOON AS WE CAN WITHIN THE CONFINES OF OUR SYSTEM.

WE HAPPEN TO BE AT AN INFLECTION POINT WHERE WE ARE CHANGING THE CONCEPT PLANS FOR A NUMBER OF OUR GRANTS, AND THIS ALLOWS US THE OPPORTUNITY, FURTHER TO MARK'S MOTION, TO FOR THE FIRST TIME MOVE UP RECONSIDERATION OF TIER III GRANTS TO A TIME EARLIER THAN WE HAVE PREVIOUSLY. AND THAT IS, WITHIN THE CONFINES OF OUR SYSTEM, THE BEST WE CAN DO. WE CAN'T GO AGAINST THE GWG WITHOUT FURTHER KNOWLEDGE ON THE PART OF THE BOARD.

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SO I BELIEVE THAT THIS IS A GOOD COMPROMISE THAT MARK HAS SUGGESTED, AND IT WILL GET BOTH OF THESE GRANTS BACK IN FOR RECONSIDERATION SHORTLY AND, BY THE WAY, WILL ALLOW FOR THEM TO TALK TO MEMBERS OF OUR TEAM WITH RESPECT TO THE CRITIQUES THAT WERE GIVEN BY MEMBERS OF THE GWG TO HELP THEM THINK THROUGH HOW TO FASHION REAPPLICATION IN A WAY THAT WILL GIVE THEM A BETTER SHOT OF APPROVAL.

SO, AGAIN, THIS IS MAINLY FOR THE BENEFIT OF OUR NEWER MEMBERS WHO ARE NOT USED TO WHAT WE HAVE HAD HERE TODAY, VERY COMPELLING STORIES AND ALL OF WHICH IS TERRIBLY IMPORTANT. SO I RECOMMEND TO THE BOARD THAT YOU ADOPT MARK'S APPROACH AS YOU DID WITH THE HUNTINGTON'S GRANT AND LET THEM REAPPLY IN SHORT ORDER.

CHAIRMAN IMBASCIANI: THANK YOU, J.T. THAT WAS ELOQUENT. NO FURTHER COMMENT, THEN I THINK WE CAN PROBABLY PROCEED TO THE VOTE.

MR. PIROVOLAKIS: AM I ALLOWED TO SAY SOMETHING MORE? AM I ALLOWED TO SAY SOMETHING MORE? AM I NOT ALLOWED TO SAY ANYTHING BEYOND THAT?

CHAIRMAN IMBASCIANI: I THINK WE PROBABLY -- HAVE WE TERMINATED PUBLIC COMMENT? BUT HE'S EXCEEDED HIS --

MR. PIROVOLAKIS: I WOULD LIKE TO SAY

SOMETHING VERY BRIEF.

CHAIRMAN IMBASCIANI: TERRY --

MR. PIROVOLAKIS: RESPECT THE BOARD --

CHAIRMAN IMBASCIANI: -- I WOULD HAVE TO DO THAT FOR 80 OTHER PEOPLE GIVEN THE RESPONSE THAT WE'VE GOTTEN FOR THIS MEETING. I'M SORRY.

MR. TOCHER: I'LL CALL THE ROLL.

MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: YES.

MR. TOCHER: JUDY CHOU.

DR. CHOU: YES.

MR. TOCHER: LEONDRA CLARK-HARVEY.

DR. CLARK-HARVEY: YES.

MR. TOCHER: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MR. TOCHER: YSABEL DURON.

MS. DURON: YES.

MR. TOCHER: MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: YES.

MR. TOCHER: ELENA FLOWERS.

DR. FLOWERS: YES.

MR. TOCHER: DAVID HIGGINS.

DR. HIGGINS: YES.

MR. TOCHER: VITO IMBASCIANI.

CHAIRMAN IMBASCIANI: YES.

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MR. TOCHER: RICH LAJARA.
MR. LAJARA: YES.
MR. TOCHER: CHRIS MIASKOWSKI.
DR. MIASKOWSKI: YES.
MR. TOCHER: ADRIANA PADILLA.
DR. PADILLA: YES.
MR. TOCHER: JOE PANETTA.
MR. PANETTA: YES.
MR. TOCHER: MARV SOUTHARD.
DR. SOUTHARD: YES.
MR. TOCHER: YAEL WYTE.
MS. WHITE: YES.
MR. TOCHER: KEVIN XU. KEVIN? WE'RE GOOD ON NUMBERS. THE MOTION CARRIES.
CHAIRMAN IMBASCIANI: OKAY. GREAT. THANK YOU VERY MUCH. AND, DR. LAM, THANK YOU SO MUCH FOR YOUR PRESENTATION AND YOUR EXPLANATIONS.
DR. DULIEGE: FIRST WANTED TO SUPPORT ENTIRELY WHAT J.T. JUST SAID ON BEHALF OF THE CIRM. AND AS THE PRESIDENT OF CIRM, I CAN SUPPORT YOU COMPLETELY. NOT ONLY ON BEHALF OF THE TEAM, BUT ON MY BEHALF AS WELL.
IT'S REALLY PARTICULARLY IMPORTANT, AND I DO AGAIN I DO THINK WE UNDERSTAND DEEPLY THE URGENCY THAT FAMILY MEMBERS AND SCIENTISTS FEEL EVERY DAY.

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WE UNDERSTAND EMOTIONALLY. I CAN'T PRETEND THAT WE'RE IN THEIR SHOES REALLY. BUT FROM A SCIENTIFIC PERSPECTIVE, I THINK YOU ALL PROBABLY WANT TO UNDERSTAND THAT HERE WE'RE GIVING ANOTHER CHANCE FOR MAYBE AN IMPROVED APPLICATION WHERE THINGS WERE NOT THAT CLEAR.

SO MY QUESTION BACK TO US NOW IS HOW MANY MONTH DELAY THIS REPRESENT, ASSUMING THAT THERE WOULD BE AN APPLICATION, LET'S SAY, THIS LATTER PROPOSAL AS SOON AS POSSIBLE?

CHAIRMAN IMBASCIANI: J.T., YOU'RE GOING TO BRING THE CONCEPT PLAN.

DR. THOMAS: I'LL TAKE IT. SO THESE ARE FURTHER TO THE NEW CLIN CONCEPT PLAN THAT WILL BE BROUGHT TO THE BOARD IN TWO MONTHS. AND ASSUMING THE BOARD APPROVES, THAT WILL IMMEDIATELY SET IN MOTION THE PROCESS OF GETTING TO WHERE APPLICATIONS WILL BEGIN TO BE ACCEPTED WHICH WILL BE IN THE SPRING AS OPPOSED TO THE WAY IT WOULD BE UNDER THE TIER III, WHICH WOULD BE IN THE SUMMER.

DR. DULIEGE: SO IT'S EFFECTIVELY A TWO-MONTH DELAY IN THE PROCESS.

DR. THOMAS: IT'S A LITTLE MORE THAN THAT, YES.

DR. DULIEGE: THREE- TO FOUR-MONTH DELAY,

WHICH --

DR. THOMAS: IT'S A COUPLE MONTHS OFF OF WHAT IT WOULD HAVE BEEN HAD THEY WAITED FOR THE NORMAL TIER III TIME.

DR. DULIEGE: OKAY. THANK YOU.

CHAIRMAN IMBASCIANI: OKAY. WE ARE NOW MOVING TO AGENDA ITEM NO. 13. THIS IS CONSIDERATION OF RECOMMENDATIONS FROM THE GOVERNANCE SUBCOMMITTEE. AND I WILL JUST PASS IT OVER TO PAT LEVITT AT THIS POINT, WHO'S ONE OF THE CO-CHAIRS OF THE SUBCOMMITTEE.

DR. LEVITT: I AM NOT JUDY GASSON, BUT I WISH I WAS AT THIS TIME.

VICE CHAIR BONNEVILLE: IN HER ABSENCE YOU MEAN?

DR. LEVITT: IN HER ABSENCE. SHE'S ON A PLANE SOMEWHERE, I THINK.

SO FOR THIS AGENDA ITEM, THE GOVERNANCE SUBCOMMITTEE MET LAST WEEK TO TAKE UP AN ITEM ABOUT THE DOCUMENTS THAT ARE USED FOR REVIEW OF THE CHAIR, VICE CHAIR, AND PRESIDENT AND CEO. AND, SCOTT, IS SUSAN WHITE ON --

MR. TOCHER: UH-HUH.

DR. LEVITT: -- WHO'S GOING TO PROVIDE THE SUMMARY OF THE MATERIALS THAT THE GOVERNANCE

COMMITTEE APPROVED UNANIMOUSLY?

MS. WHITE: YES. AND, PAT, I'M HERE. I DON'T KNOW IF YOU CAN SEE ME. HELLO.

DR. LEVITT: WE CAN HEAR YOU.

MS. WHITE: OKAY. PERFECT. EXCELLENT. SO THANK YOU. IT'S WONDERFUL TO BE BACK WITH ALL OF YOU, AND I WANTED TO GIVE YOU A QUICK UPDATE ON THE EVALUATION OF THE THREE EXECUTIVE POSITIONS THAT WE'RE GOING TO BE DOING IN 2025.

SO WE ARE GOING TO DO THE CHAIR AND THE VICE CHAIR OF THE BOARD AGAIN, AND WE'RE GOING TO USE THE SAME QUESTIONS THAT WERE ASKED LAST YEAR. AND WE'RE ALSO GOING TO BE DOING ONE FOR J.T., SO THE PRESIDENT AND CEO POSITION. AND THAT WILL BE A NEW ONE. SO THOSE OF YOU WHO HAVE BEEN THE BOARD LAST YEAR AND ARE STILL THERE, WE'RE GOING ASK THE SAME QUESTIONS FOR MARIA AND VITO, BUT ON J.T. WE USED THE SEARCH CRITERIA, THE COMPETENCY STANDARD OF THE JOB TO FORMULATE THE SURVEY QUESTIONS.

SO THE TIMELINE, I DID WANT TO GIVE YOU A QUICK HEADS UP ON IT. SO LET ME SEE IF I CAN SHARE MY SCREEN. LET ME GO AHEAD AND SEE IF I CAN GET THAT A LITTLE BIT BIGGER. YEAH.

SO WE ARE GOING TO DO THE SAME PROCESS --

DR. LEVITT: WE CAN'T SEE THE DOCUMENT.

THERE YOU GO. OKAY.

MS. WHITE: OH, GOOD. WE'RE GOING TO USE THE SAME PROCESS WHERE VITO, MARIA, AND J. T. --

MR. TOCHER: SUSAN, SORRY. THIS IS SCOTT. IS THERE ANY WAY YOU COULD SHOW SINGLE PAGE AT A TIME? IT'S AWFULLY SMALL ON OUR TEENY TINY LITTLE MONITORS ALTHOUGH I'D ENCOURAGE PEOPLE TO LOOK INTO THEIR ELECTRONIC NOTEBOOKS IF THEY HAVE IT. IT'S ATTACHED ON OUR AGENDA.

MS. WHITE: YES. YOU KNOW WHAT I MIGHT DO. CLAUDETTE, I THINK, HAS THE -- WHY DON'T I LET HER SHOW THE SCREEN. SHE'LL BE ABLE TO -- IF I STOP SHARING, CLAUDETTE MIGHT SHARE THE SCREEN SO SHE CAN DO IT.

DR. LEVITT: IT'S IN YOUR ELECTRONIC NOTEBOOK. IT'S THE BRIEFING BOOKS. GO AHEAD, SUSAN. THERE YOU GO. PERFECT.

MS. WHITE: OH, GOOD. I'M SO GLAD.

SO WE'RE GOING TO ASK EACH OF THE THREE INCUMBENTS TO DO A SELF ASSESSMENT, AND THEY'RE GOING TO BE ASSESSING THEMSELVES ON THE SURVEY BETWEEN MAY 23D AND JUNE 2D. AND THEN WE WILL GET ALL OF YOU A COPY OF THEIR SELF-ASSESSMENT RESULTS SO YOU HAVE A CHANCE TO SEE WHAT THEY FEEL THEIR CONTRIBUTIONS WERE AND HOW THEY DID. AND THEN

YOU'LL BE ANSWERING THE SURVEY, YOUR SURVEY QUESTIONS AFTER LOOKING AT THEIRS JUNE 5TH TO JUNE 16. AND THEN I WILL BE COMPILING ALL THE INFORMATION, ANALYZING IT, AND DEVELOPING A REPORT, A SUMMARY, OF ALL OF YOUR FEEDBACK ON THE SURVEY AS WELL AS INTERVIEWS THAT WE'RE GOING TO BE DOING.

I WILL BE INTERVIEWING ALL OF THE SUBCOMMITTEE CHAIRS AND CO-CHAIRS AS WELL AS ALL THE DIRECT REPORTS FOR THOSE THREE POSITIONS. SO PEOPLE WILL HAVE A CHANCE TO VERBALLY ALSO GIVE FLAVOR, COLOR, BACKGROUND, ADDITIONAL COMMENTS.

IF ANY OF THE BOARD MEMBERS, AS ALWAYS, WOULD LIKE TO HAVE AN INTERVIEW WITH ME, YOU'RE MORE THAN WELCOME. AND SOME PEOPLE DO TAKE ADVANTAGE OF THAT, MOST DO NOT. IT'S ENTIRELY UP TO YOU. SO I WILL HAVE THE SUMMARY OF THE INTERVIEWS, THE THINGS THAT WE HEAR, YOUR SURVEY DATA RESULTS, AND FOR MARIA AND VITO WE'LL BE ABLE TO COMPARE AGAINST LAST YEAR. I'LL HAVE ALL THAT READY FOR PAT AND JUDY AND THE BOARD GOVERNANCE SUBCOMMITTEE AUGUST THE 5TH.

SO AFTER THAT, RIGHT NOW THE PLAN -- I'LL JUST SCROLL DOWN A LITTLE BIT HERE. THE PLAN IS THAT WE'LL BE AT THE SEPTEMBER BOARD MEETING AGAIN WHERE WE HAVE AN OPPORTUNITY THIS TIME TO GET THE RESULTS FOR ALL THREE OF THE EXECUTIVE POSITIONS'

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PERFORMANCE. AND YOU WILL DISCUSS SHARING THAT INFORMATION WITH THE INDIVIDUALS.

I'M TRYING TO THINK. IS THERE ANYTHING ELSE OR ANY QUESTIONS PEOPLE MIGHT HAVE? I'D BE HAPPY TO GO THROUGH THE DETAIL. J.T.'S IS THE NEWEST ONE, AND I'D BE GLAD TO GO THROUGH THAT IF ANYONE WOULD LIKE ME TO IN GREAT DETAIL OR NOT, WHATEVER YOUR PREFERENCE.

DR. LEVITT: SO, SUSAN, IT'S PAT. FIRST, I SHOULD HAVE INTRODUCED, SUSAN WHITE, WHO'S THE CONSULTANT AND HAS BEEN WORKING WITH US ON REVIEWS, I DON'T REMEMBER, AT LEAST ONE YEAR, MAYBE MORE, LONGER.

MS. WHITE: THIS IS THE THIRD YEAR.

DR. LEVITT: AND SO FOR NEW BOARD MEMBERS, SUSAN HAS WORKED VERY CLOSELY WITH JUDY, MYSELF, AND THE GOVERNANCE COMMITTEE ON THE REVIEWS. YOU ALL HAD THE -- SO THE ONE CHANGE, THE ONE SIGNIFICANT CHANGE IS TO ALIGN THE REVIEW OF THE PRESIDENT AND CEO IN TERMS OF CONTENT OF WHAT'S BEING REVIEWED. THERE'S SOME SPECIFIC THINGS FOR EACH OF THE POSITIONS. BUT THE NUMBER OF QUESTIONS ARE GREATLY REDUCED, AND THERE'S ALIGNMENT NOW WITH ALL THREE POSITIONS IN TERMS OF THE FORMS THAT WE'RE GOING TO BE FILLING OUT. SO FROM THE GOVERNANCE SIDE OF THE

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REPORT, I THINK THE GOVERNANCE SUBCOMMITTEE HAD SOME GOOD DISCUSSION. IT WAS THE ALIGNMENT THAT THEY WERE VERY HAPPY TO SEE, CONSISTENCY, AND THERE WAS VERY LITTLE DISCUSSION. THERE WAS UNANIMOUS APPROVAL.

SO I HAVE TO DO A MOTION. I'D ASK FOR A MOTION TO APPROVE THE REVIEW FORMS AND THE TIMELINE FOR REVIEW OF THE BOARD CHAIR, VICE CHAIR, AND PRESIDENT AND CEO. DO I HAVE A MOTION?

DR. DEAS: SO MOVED.

DR. BLUMENTHAL: SECOND.

DR. LEVITT: DEBORAH AND GEORGE.

MR. TOCHER: WE GOT IT.

DR. LEVITT: YOU DISTRACT ME FROM MY NOTES.

VICE CHAIR BONNEVILLE: OF COURSE.

DR. LEVITT: SO COMMENTS OR QUESTIONS, ANYBODY? OKAY. SCOTT, CALL THE ROLL.

MR. TOCHER: JUST LOOK FOR ANY PUBLIC COMMENT IN THE ROOM.

DR. LEVITT: ANY PUBLIC COMMENT? I LOOKED DIRECTLY AT CLAUDETTE AND SHE JUST WENT LIKE THIS.

MR. TOCHER: OR ONLINE. OKAY. GREAT. I'LL CALL THE ROLL. ACTUALLY I WON'T CALL THE ROLL FOR THE ROOM. ALL THOSE IN THE ROOM IN FAVOR SAY

AYE. OPPOSED SAY NAY. ANY ABSTENTIONS? AND I'LL POLL THE MEMBERS ON THE ZOOM.

JUDY CHOU.

DR. CHOU: AYE.

MR. TOCHER: DAVID HIGGINS. SORRY, DAVID, ARE YOU STILL -- I SEE YOU. I'LL COME BACK TO YOU, DAVID.

RICH LAJARA.

MR. LAJARA: YES.

MR. TOCHER: ADRIANA PADILLA. JOE PANETTA. SORRY, JOE.

MR. PANETTA: YES.

MR. TOCHER: KEVIN XU. KEVIN, PERHAPS YOU'RE MUTED. KEITH YAMAMOTO.

DR. YAMAMOTO: YES.

MR. TOCHER: THANK YOU. LET ME JUST COME BACK TO DAVID HIGGINS.

DR. HIGGINS: YES.

MR. TOCHER: THANK YOU, DAVID. AND LAST CALL FOR KEVIN XU. VERY GOOD. OKAY. THANK YOU. THE MOTION CARRIES.

DR. LEVITT: MOTION CARRIES. THANKS VERY MUCH. OKAY. NOW WE'RE GOING TO BE ENTERING A CLOSED SESSION. SCOTT, YOU HAVE YOUR FINGER ON PAUSE.

MR. TOCHER: NO. NO.

DR. LEVITT: I WAS GOING TO ASK YOU TO READ THE STATUTE THAT SENDS US INTO CLOSED SESSION.

MR. TOCHER: OKAY. I SHOULD HAVE THIS MEMORIZED BY NOW. THE BOARD WILL BE ADJOURNING TO CLOSED SESSION FOR A DISCUSSION OF PERSONNEL PURSUANT TO GOVERNMENT CODE SECTION 11126 (A) AND HEALTH AND SAFETY CODE SECTION 125290.30(F)(3)(D).

SO OUR MEMBERS WHO ARE ON THE ZOOM WILL SEE A TAB TO INVITE YOU TO JOIN A BREAKOUT ROOM. SO CLICK THE BLUE BUTTON TO JOIN THAT BREAKOUT ROOM. MEMBERS OF THE PUBLIC IN THIS ROOM AND THE TEAM WILL LEAVE US JUST THE BOARD.

(THE BOARD THEN WENT INTO CLOSED SESSION, NOT REPORTED, NOR HEREIN TRANSCRIBED. THE FOLLOWING WAS THEN HEARD IN OPEN SESSION.)

DR. LEVITT: WELCOME BACK FROM THE BREAK AND JUST TO REPORT BACK THAT NO ACTIONS WERE TAKEN IN THE CLOSED SESSION. THANK YOU.

CHAIRMAN IMBASCIANI: BACK TO ME? OKAY. ARE ALL THE BOARD MEMBERS HERE?

WELL, PAT, IF I COULD JUST FOOTNOTE ONTO YOUR COMMENT. I'VE GOT TO SHARE WITH BOARD MEMBERS IT ACTUALLY IS QUITE STRANGE TO COME BACK INTO A ROOM WHERE YOU KNOW EVERYONE HAS BEEN TALKING ABOUT

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YOU.

FOR THE CATHOLICS IN THE ROOM, THEY MIGHT -- IT WILL BE RESONANT WITH, YOU KNOW, THERE ARE OTHER OPPORTUNITIES FOR CATHOLICS TO DISCUSS THEIR SHORTCOMINGS AND ASPIRATIONS. IT'S CALLED CONFESSION. BUT THE DIFFERENCE IS THAT'S CONDUCTED IN A CLOSED ROOM ONE ON ONE AND IT'S VERY DARK.

DR. LEVITT: I THINK BECAUSE I SAID THERE'S NO ACTION, I THINK WE SHOULD MOVE ON. THAT'S MY RECOMMENDATION.

CHAIRMAN IMBASCIANI: SO I SEE THE PRESIDENT IS IN THE BATTING CAGE HERE, READY TO GO. THE PRESIDENT'S REPORT THE NEXT AGENDA ITEM.

DR. THOMAS: THANK YOU. WITH PERHAPS SOME LIMITED EXCEPTION OF PUBLIC COMMENT AND THAT SORT OF THING, I'VE BEEN INFORMED I'M THE FINAL THING BETWEEN GETTING TO GO HOME, ET CETERA. SO I KNOW MARIA IS WATCHING CLOSELY.

VICE CHAIR BONNEVILLE: I AM.

DR. THOMAS: AND SCOTT ALREADY PUT THE TIMER ON.

SO, MR. CHAIR, MADAM VICE CHAIR, MEMBERS OF THE BOARD, MEMBERS OF THE PUBLIC, THANK YOU FOR THE OPPORTUNITY TO PLAY CLEANUP HERE IN THIS MEETING. I'VE GOT A COUPLE THINGS I WANT TO TALK

ABOUT SPECIFICALLY, BUT I DO WANT TO START WITH A COMMENT OR TWO ABOUT THE LAST FEW WEEKS IN LOS ANGELES, WHICH HAVE BEEN EXTRAORDINARILY TAXING WHERE MANY PEOPLE AT A NUMBER OF OUR INSTITUTIONS LOST THEIR HOMES EITHER IN ALTADENA OR THE PALISADES. MANY OTHERS AFFECTED IN A GREAT MANY WAYS, WHETHER IT WAS SMOKE DAMAGE OR VERY LENGTHY EVACUATIONS WHICH BY COMPARISON ARE MINOR, MINOR, MINOR INCONVENIENCES, BUT IT WAS A REALLY DIFFICULT TIME.

AND WE HEARD FROM A NUMBER OF OUR RESEARCHERS THAT THE DISRUPTION REALLY HAD AN IMPACT ON A NUMBER OF LABS AND PEOPLE'S ABILITY TO GET IN AND OUT OF PLACES AND THAT SORT OF THING. AND I WANTED TO REPORT TO THE BOARD THAT WERE WE COULD WE REACTED TO THAT TO ACCOMMODATE THESE DISRUPTIONS. FOR EXAMPLE, WE HAD OUR LAST DISC-0 APPLICATION DEADLINE WAS EARLY FEBRUARY. AND WE WERE INFORMED THAT A NUMBER OF PEOPLE WHO WERE PUTTING TOGETHER GRANTS FOR THE DISC-0S WERE AFFECTED AND UNABLE TO GET INTO THEIR LABS. THEY COULDN'T GET TO A LOT OF THE DATA THAT WAS GOING TO INFORM THEIR APPLICATIONS. AND SO WE TALKED ABOUT IT INTERNALLY AND FELT WE ABSOLUTELY NEEDED TO DO OUR PART TO HELP THEM OUT.

SO WE EXTENDED THE APPLICATION BY TWO MONTHS, WHICH WAS RECEIVED VERY FAVORABLY BY OUR MEMBER INSTITUTIONS. BUT JUST A SMALL PART OF THINGS THAT PEOPLE HAVE DONE TO TRY TO HELP. SO I JUST WANTED TO REPORT THAT TO THE BOARD. AND HAPPY TO SAY THAT WITH THE BIT OF RAIN WE HAD LAST WEEKEND, WE'RE PRETTY MUCH THROUGH THAT STRETCH, AND WE'RE HOPING THAT THE NEXT SET OF FIRE CONDITIONS THAT UNDOUBTEDLY WILL ARISE SHORTLY WILL PASS WITHOUT INCIDENT. AND I JUST WANT TO SAY PERSONALLY, AS SOMEBODY WHO LIVES IN THE WEST SIDE OF LOS ANGELES, THAT THE FIREWOMEN AND MEN ARE HEROES. THEY PERFORMED INCREDIBLY UNDER EXTREMELY ADVERSE CONDITIONS, ALMOST UNPRECEDENTED AT THE OUTSET, AND WERE ABLE TO SAVE A GREAT DEAL OF PROPERTY AND PEOPLE'S LIVES AND ET CETERA. SO A MAJOR SHOUT OUT TO THE FIRE DEPARTMENT, NOT JUST OF L.A., BUT LITERALLY THERE WERE FIREMEN AND WOMEN COMING FROM ALL AROUND THE COUNTRY TO HELP OUT. AND THEY JUST DID YEOMAN WORK, AND WE'RE ENDLESSLY APPRECIATIVE.

OKAY. SO WITH THAT, I WANT TO GO INTO THE PRESIDENT'S REPORT SO I'M GOING TO TALK TO YOU ABOUT A COUPLE THINGS. ONE IS, AS YOU KNOW, AT THE BEGINNING OF EACH CALENDAR YEAR, THERE'S THE MASSIVE

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JP MORGAN CONFERENCE OF THE BIOTECH INDUSTRY IN SAN FRANCISCO. IT PRETTY MUCH TAKES OVER THE CITY. AT THE BEGINNING OF THAT WEEK, WHICH IMMEDIATELY SUCCEEDED THE ISSCR NUCLEUS FORUM THAT VITO DESCRIBED EARLIER IN THE MEETING, THE ALLIANCE FOR REGENERATIVE MEDICINE, WHICH IS SORT OF A ASSOCIATION FOR OUR INDUSTRY, GIVES A STATE OF THE INDUSTRY REVIEW, WHICH SORT OF SETS CONTEXT GOING INTO EACH YEAR FOR WHAT'S GOING ON IN CELL AND GENE THERAPY FROM THE BUSINESS SIDE.

SO I'M GOING TO GO THROUGH A NUMBER OF SLIDES BECAUSE I THINK THESE ARE INTERESTING, HOPEFULLY, EVEN LATE IN THE DAY AND WILL GIVE YOU SOME DATA POINTS THAT YOU CAN TAKE AWAY FROM THE MEETING HERE.

SECONDLY, I'LL VERY BRIEFLY RECAP THE AFOREMENTIONED MEETING WE HAD WITH THE STATE CONTROLLER AND THE CITIZENS FINANCIAL ACCOUNTABILITY OVERSIGHT COMMITTEE IN SACRAMENTO IN DECEMBER.

SO WITHOUT FURTHER ADO, PLUNGE INTO THE ARM PRESENTATION. SO ECHOING THE SORT OF POSITIVE SENTIMENT THAT EMANATED FROM THE NUCLEUS FORUM, WE HAD A PRETTY UPBEAT PRESENTATION BY TIM HUNT, WHO'S THE CEO OF ARM, WHO A NUMBER OF US HAVE A GOOD RELATIONSHIP WITH WHO'S VERY MUCH A GREAT AMBASSADOR

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FOR THE INDUSTRY. AND HIS PRESENTATION WAS ALL ABOUT HOW THINGS ARE LOOKING UP FROM, AGAIN, PRINCIPALLY THE BUSINESS SIDE.

SO HE BROKE DOWN HIS PRESENTATION INTO FOUR SECTIONS. HISTORY AS A GUIDE. THIS LOOKS LIKE A JEOPARDY BOARD, BY THE WAY. HISTORY AS A GUIDE, ADDRESSING QUESTIONS ABOUT CELL AND GENE THERAPY COMMERCIALIZATION, THE INCREDIBLY BRIGHT FUTURE OF CELL AND GENE THERAPY, AND THEN THE SORT OF INTERESTING FOURTH, OPPORTUNITIES IN THE NEW ADMINISTRATION. WE'LL GET TO THAT ONE AT THE END.

SO HISTORY AS A GUIDE. SO I'M NOT GOING TO GO THROUGH THIS OTHER THAN TO NOTE THAT HIS POINT WAS FROM THE FIRST RESEARCH DONE IN MONOCLONAL ANTIBODIES TO WHEN THE FIRST PRODUCT WAS APPROVED WAS 23 YEARS. AND THE POINT OF THIS IS THAT WITH EACH SORT OF ADVANCEMENTS IN THE NEW AREA OF SCIENCE, IT TAKES TIME TO GET TO WHERE YOU GO FROM FIRST INCEPTION TO COMMERCIALIZATION AND THEN THINGS PROCEED FROM THERE. OF COURSE, MONOCLONAL ANTIBODIES ARE NOW A HUGE PART OF THE MARKET. YOU SEE $250 BILLION IN REVENUE. POINT BEING THAT SIMILAR SORT OF THING APPLIES TO CELL AND GENE THERAPY WHERE THE FIRST GENE THERAPY CONCEPT CAME IN 1972, AND IT WASN'T REALLY UNTIL YOU GOT INTO THE

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LATE 2010S THAT YOU STARTED TO SEE APPROVALS OF CELL AND GENE THERAPY PRODUCTS.

YOU CAN SEE IN 2019 SCOTT GOTLIEB, WHO IS THE FDA COMMISSIONER IN THE FIRST TRUMP ADMINISTRATION, PREDICTED THAT YEAR THAT BY 2025 WE'D HAVE 10 TO 20 APPROVALS A YEAR. AND AS YOU SORT OF GO THROUGH AND YOU SEE A COUPLE OF THE BIG NAMES, ZOLGENSMA, WHICH WAS NOVARTIS' SMA PRODUCT, APPROVED AND BECAME A BILLION-DOLLAR BLOCKBUSTER IN 2021.

YESCARTE, WHICH IS A CAR T PRODUCT FROM KITE THERAPEUTICS, LATER ACQUIRED BY GILEAD FOLLOWED SUIT IN 2023 -- SORRY -- YES -- 2023. ALSO IN 2023 FIVE FDA APPROVALS IN THE CELL AND GENE THERAPY SPACE, NINE LAST YEAR. SO WHEN YOU GET TO 2025, WHERE WE ARE NOW, HIS PREDICTION OF 10 TO 25 APPROVALS A YEAR IS NOT TOO FAR AFTER THE MARK.

OKAY. SO THEN QUESTIONS ABOUT -- THIS IS A BIG TOPIC, CELL AND GENE THERAPY COMMERCIALIZATION, WHICH GETS -- DOVETAILS VERY NICELY WITH MADAM VICE CHAIR'S AND MR. LOMAX AND THE ACCESS AND AFFORDABILITY WORKING GROUP, AND THE PATIENT ACCESS TEAMS, THIS ISSUE OF COMMERCIALIZATION, ACCESS AND AFFORDABILITY IS A BIG DEAL.

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THE SLIDES ON THIS, THIS SLIDE JUST IS TO SAY THAT THERE ARE A NUMBER OF ISSUES IN THE FIELD AND THINGS AREN'T PERFECT NOW. THERE ARE THINGS THAT HAVE TO BE WORKED THROUGH. SO YOU CAN SEE SOME THERAPIES FACE CHALLENGES RELATED TO EFFICACY, SAFETY, AND DURABILITY. YOU'VE GOT COST ISSUES. YOU'VE GOT ALL SORTS OF COMMERCIALIZATION ELEMENTS THAT ARE TRICKY, ALL LISTED HERE WHICH YOU CAN READ. I NEEDN'T GO THROUGH. BUT THE POINT BEING IS THERE ARE A LOT OF THINGS THAT NEED TO BE RESOLVED IN THE COMMERCIALIZATION FRONT, WHICH ARE A WORK IN PROGRESS AT THE MOMENT.

THERE HAVE BEEN SOME NOTABLE SUCCESSES. SO YOU HAD VERTEX WITH CASGEVY, A TREATMENT FOR SICKLE CELL. FOR THOSE OF US WHO WENT TO THE MEETING ON THE MESA IN PHOENIX, WE SAW THIS REMARKABLE PATIENT JIMMY OLAGHERE WHO HAD SEVERE SICKLE CELL DISEASE, TOOK CASGEVY IN 2020 AND SPOKE ABOUT HIS FOUR YEARS LATER CLIMBING TO THE TOP OF MOUNT KILIMANJARO, WHICH IS A VERY INSPIRING STORY. SO IT'S SOMETHING THAT THINGS ARE HAPPENING IN THE SPACE AND PRODUCTS ARE REACHING COMMERCIALIZATION AND HAVING REAL IMPACT.

OKAY. SO COMMERCIALIZATION, QUESTION 1: DO CELL AND GENE THERAPIES REPRESENT COMPELLING

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COMMERCIAL OPPORTUNITIES? SO HERE THIS IS SUPPOSED TO SAY THAT, AS I MENTIONED BEFORE, THERE HAVE BEEN SEVERAL BLOCKBUSTERS NOW IN THE FIELD DEFINED AS A BILLION OR MORE REVENUE A YEAR. THEY PREDICT BY THE YEAR 2030 THERE WILL BE TEN OF THOSE AT LEAST. AND YOU GO TO THE NEXT PAGE, THIS IS SORT OF THE NEXT TIER DOWN, 500 MILLION PLUS IN ANNUAL REVENUES. YOU HAD TEN SUCH COMPANIES IN 2024. BY 2030 THEY EXPECT TO BE 50 SUCH COMPANIES, WHICH WOULD BE A REAL INCREASE BY ANY ESTIMATION.

PROMISING MARKETS FOR BLOCKBUSTER POTENTIAL. SO HERE WE'VE GOT SICKLE CELL, DUCHENNE'S, DYSTROPHIC EPIDERMOLYSIS BULLOSA, AND DANNON DISEASE. AND YOU CAN SEE THE NUMBER OF PATIENTS THAT ARE POTENTIALLY IN THE MARKET AND BELOW, THE VERY BOTTOM ROW, THE COMPANIES THAT ARE INVOLVED IN DEVELOPING THERAPIES IN THAT FIELD.

QUESTION NO. 2: LARGE-CAP BIOPHARMA COMPANIES INVESTING IN CELL AND GENE THERAPIES. SO IF YOU GO BACK, I'D SAY, FIVE YEARS OR SO, THE LIST WAS PRETTY SMALL BECAUSE THEY'RE TAKING A WAIT-AND-SEE SORT OF ATTITUDE AS TO WHAT WAS HAPPENING IN THE RESEARCH, WHAT WAS GOING TO BE VIABLE, ET CETERA. AND IF YOU NOW GO, OF THE TOP 15 BIG BIOPHARMA, 13 OF THOSE ARE IN THE CELL AND GENE

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THERAPY SPACE IN ONE WAY OR ANOTHER WITH MERCK AND AMGEN BEING THE HOLDOUTS. AND THEN WE'LL SEE IN A YEAR OR TWO IF EVEN THEY JOIN THE FRAY AS WELL. I THOUGHT THIS WAS A VERY INTERESTING SLIDE, THIS ONE.

OKAY. SO LARGE-CAP BIOPHARMA CAN DRIVE COMMERCIAL SUCCESS VIA PARTNERSHIPS AND ACQUISITIONS. AND HERE'S A LIST OF GROUPS THAT CAME TOGETHER. INTERESTINGLY ENOUGH, MOST RECENTLY IN THE BOTTOM RIGHT ROCHE ACQUIRING POSEIDA, POSEIDA BEING A CIRM GRANTEE, BUT YOU SEE IN THE VARIOUS INDICATIONS LISTED THERE, THAT THERE'S SOME PRETTY POWERHOUSE COMBOS, AND THEY'RE REALLY DRIVING COMMERCIAL SUCCESS ACROSS A NUMBER OF INDICATIONS.

QUESTION 3: IS CELL AND GENE THERAPY SOLELY IN THE U.S.? OF COURSE, WE KNOW THE ANSWER TO THAT IS NO. BUT HERE'S A BIT OF DETAIL WHICH SHOWS THAT 35 PERCENT OF REVENUE IS EX-U.S. AND IF YOU BREAK THAT DOWN, THERE ARE A NUMBER OF THESE BIG-TIME PRODUCTS THAT ARE OUT THERE THAT A MAJORITY OF THE SALES ARE OUTSIDE THE U.S. SO CELL AND GENE THERAPY IS PERMEATING THE INTERNATIONAL LANDSCAPE.

HERE'S JUST ONE STORY. AGAIN, ZOLGENSMA, NOVARTIS' SPINAL MUSCULAR ATROPHY PRODUCT, NOW BEEN APPROVED IN 50 PLUS COUNTRIES. THE ACCESS PATH ESTABLISHED IN OVER 45 OF THOSE. YOU CAN SEE THAT

IT'S NOW OVER 4,000 YOUNG CHILDREN HAVE BEEN TREATED WORLDWIDE AND THAT THEY'RE IN PHASE 3 TRIALS FOR APPLICATION UP TO AGE 18, BLOCKBUSTER STATUS OF A BILLION IN 2021, EXPECTED TO GROW TO 2 BILLION BY 2028. AND THEN ESTABLISHING ACCESS CAPABILITIES EARLY, WHICH IS ONE THING WE TALK ABOUT A LOT IN-HOUSE, IS CRITICAL FOR COMMERCIALIZATION, WHICH IS ONE OF THE LESSONS LEARNED FROM THE ZOLGENSMA STORY.

OKAY. THIS SEGMENT OF HIS TALK WAS THE INCREDIBLY BRIGHT FUTURE OF CELL AND GENE THERAPY. AND WE GET INTO SOME STATS HERE. U.S. CONTINUES TO LEAD, SECTORS GLOBALIZING. YOU CAN SEE PRODUCT, THE COMPANIES DEVELOPING PRODUCT UP 6 PERCENT FROM LAST YEAR, NUMBER OF CLINICAL TRIALS UP 3 PERCENT, AND THE AMOUNT OF INVESTMENT, WHICH IS INTERESTING, UP 30 PERCENT, WHICH IS A VERY HEFTY ADD.

YOU SAW A LOT OF DIFFERENT WAYS OF FINANCING AND ACQUISITION AFFECTING THE SPACE. YOU HAVE THE IPO EXAMPLES ON THE LEFT, WHICH WERE SIZABLE. YOU HAD VERY LARGE VC ROUNDS IN THE CENTER, ALSO SIZABLE. ACQUISITION AMOUNTS, THERE AGAIN, ROCHE ACQUIRING POSEIDA, OUR GRANTEE, FOR A BILLION AND A HALF, VERY SIGNIFICANTLY SIZED DEALS.

THIS IS AN INTERESTING PAGE, I THINK. AS

YOU KNOW, 50 PERCENT OF OUR PORTFOLIO IS IN RARE DISEASE. WE REMAIN EXTREMELY COMMITTED TO THE RARE DISEASE SPACE. THIS PARTICULAR SLIDE IS OF INTEREST BECAUSE IT TALKS ABOUT POTENTIAL PREVALENT DISEASE BREAKTHROUGHS THAT ARE COMING IN WET AMD, PARKINSON'S, MULTIPLE SCLEROSIS, AND TYPE 1 DIABETES. MANY OF THESE COMPANIES WE'VE BEEN IN DISCUSSIONS WITH TO VARIOUS EXTENTS, NOT THAT THEY NECESSARILY ARE ACTIVE IN CALIFORNIA, BUT WE'RE VERY PLUGGED INTO THE NETWORKS HERE.

ONE OF THE PRINCIPAL SCIENTISTS, LORENZ STUDER BEHIND THE PARKINSON'S WORK AT BLUEROCK AND BAYER WAS AT THE NUCLEUS FORUM. A LOT OF US HAD A GOOD OPPORTUNITY TO CATCH UP WITH LORENZ TO HEAR THE LATEST AND GREATEST. VERTEX AND THE TYPE 1 DIABETES HAS ACQUIRED DOUG MELTON'S COMPANY, SEMMA THERAPEUTICS AND ALSO ACQUIRED VIACYTE AS YOU WILL RECALL. DOUG IS A VERY CLOSE FRIEND, AND WE TALK TO HIM AT FAIRLY REGULAR INTERVALS ABOUT THE PROGRESS THAT HE CAN TALK ABOUT PUBLICLY, BUT THEY'RE MAKING GREAT STRIDES. BUT YOU CAN SEE THAT IN THE NOT TOO DISTANT FUTURE THERE ARE GOING TO BE SOME MAJOR DEVELOPMENTS OF SOME VERY BIG-TIME PREVALENT DISEASES.

A NUMBER OF TRENDS DRIVING THE

ADVANCEMENTS IN SOLID TUMOR. CAR-T STARTING TO GET INTO SOLID TUMOR IN A BIG WAY. IT'S ALSO STARTING TO GET INTO AUTOIMMUNE AND LUPUS AND A NUMBER OF OTHER INDICATIONS. AND THEN YOU'RE STARTING TO SEE SOME REAL INTERESTING WORK IN-VIVO GENE EDITING WHICH IS CLEARLY GOING TO BE ONE OF THE WAVES OF THE FUTURE, AND WE'LL SEE A LOT MORE OF THAT AS TIME GOES BY.

OKAY. NOW WE GET TO THIS PART OF THE PRESENTATION. SO WE HAVE PICTURES OF OUR TWO MOST RECENT PRESIDENTS. THE POINT OF THIS SLIDE WAS EVERY PRESIDENCY BRINGS BOTH HEADWINDS AND TAILWINDS. AND YOU CAN SORT OF LOOK AND SEE WHAT THEY LISTED WITH RESPECT TO THE FIRST TRUMP ADMINISTRATION AND THE BIDEN ADMINISTRATION.

AND THE ISSUE WAS THAT NO MATTER WHO'S PRESIDENT, THERE ARE GOING TO BE CHALLENGES AND OPPORTUNITIES. THIS WAS PRESENTED, AS I MENTIONED, EARLY JANUARY, PRECEDED A NUMBER OF THE MORE RECENT EXECUTIVE ORDERS AND OTHER COMMENTARIES.

SO TIM TOOK AT STAB AT HOW THINGS UNDER TRUMP ADMINISTRATION 2.0 MAY BE THINGS THAT CELL AND GENE THERAPY CAN BENEFIT FROM. ONE WAS THAT HAVING THERAPIES AND CURES THAT GET RID OF THE DISEASES AND REDUCE THE NEED FOR CHRONIC CARE IS A MAJOR PILLAR

OF THE CURRENTLY UNDER CONSIDERATION SECRETARY OF HHS NOMINEE'S MAKE AMERICA HEALTHY AGAIN AGENDA AND SHOULD BE IN SYNC WITH THAT PHILOSOPHICALLY.

THE SECOND POINT WAS THAT THERE'S GOING TO BE REGULATORY FLEXIBILITY THAT ALLOWS FOR ACCELERATED APPROVALS, ET CETERA, WITH NEW TECHNOLOGIES. I'M NOT EXACTLY SURE HOW WE CAN PREDICT HOW THAT'S GOING TO GO, BUT VERY HOPEFULLY THAT'S GOING TO BE THE CASE THAT CLEARLY IS WHERE PETER MARKS AND CBER IS INTENDING TO TAKE RARE DISEASE, IF HE'S ABLE TO DO SO, WHICH WOULD BE A MAJOR DEVELOPMENT FOR THAT PART OF THE DISEASE SPECTRUM.

NO. 3 IS, AND AGAIN THIS IS SOMETHING THAT REMAINS TO BE SEEN ALSO, CONTINUATION OF CMMI'S CELL AND GENE THERAPY ACCESS MODEL WHICH IS, FOR THOSE WHO AREN'T ENTIRELY FAMILIAR WITH, IMPROVES HEALTH OUTCOMES FOR PEOPLE ON MEDICAID WHO CAN BENEFIT FROM CELL AND GENE THERAPY BY SUPPORTING OUTCOME-BASED AGREEMENTS BETWEEN STATES AND MANUFACTURERS THAT WILL PROVIDE FOR TREATMENTS WITHIN A FRAMEWORK THAT LOWERS PRICES FOR STATES AND IS TIED TO PAYMENT OUTCOMES.

AND THE TWO COMPANIES THAT RECENTLY HAD THEIR STEM CELL PRODUCTS APPROVED HAVE SIGNED ONTO

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THIS IN CONCEPT, AND THERE'S HOPE THAT THIS IS A MODEL FOR TREATING THESE SORTS OF THINGS WITH MANY MORE COMPANIES IN THE FUTURE.

NO. 4, STRENGTHENING MANUFACTURING CAPACITY IN THE U.S. AND ONSHORING AS MUCH OF THE TECHNOLOGY AS POSSIBLE TO FURTHER THE AMERICA FIRST AGENDA OF THIS ADMINISTRATION. AND THEN THERE'S THE SENTIMENT THAT HAVING A NEW FTC COMMISSIONER IS GOING TO BE A BOON FOR M&A IN GENERAL AND THAT THAT SHOULD INURE TO THE BENEFIT OF THE INDUSTRY AS IT CONTEMPLATES SMALLER FIRMS BEING MERGED INTO OR BOUGHT OUT BY THE BIG PHARMA.

SO THOSE WERE TIM'S FIVE TOP IDEAS OF HOW THINGS COULD GEL WITH THE PHILOSOPHY OF PRESIDENT TRUMP. AND, AS I SAY, WE'LL SEE HOW ALL THIS PLAYS OUT.

SO SORT OF THE KEY TAKEAWAYS FROM TODAY BEING FIRST DAY OF JP MORGAN WEEK, HISTORY PROVIDES A GUIDE, CELL AND GENE THERAPIES ARE FOLLOWING A WELL-ESTABLISHED LINEAR PATH -- OR NONLINEAR PATH TOWARDS GREATER ADOPTION MUCH LIKE MONOCLONAL ANTIBODIES DID. AND IF YOU WENT BACK, SMALL MOLECULES BEFORE THAT. BLOCKBUSTERS BECKON. WE'VE GOTTEN A COUPLE BIG ONES ALREADY. WE EXPECT TO HAVE A NUMBER MORE IN THE NEXT FIVE YEARS. SCIENTIFIC

BREAKTHROUGHS CONTINUE APACE. AGAIN, YOU ARE GOING TO SEE LOTS OF THINGS LIKE IN-VIVO GENE EDITING AND A VARIETY OF OTHER DEVELOPMENTS THAT FUNDAMENTALLY AFFECT THE FIELD. AND THEN SYSTEMS MODERNIZING THROUGH THINGS LIKE THE CELL AND GENE THERAPY ACCESS MODEL AND OTHER OPPORTUNITIES.

AND THAT WAS THAT. THAT WAS TIM HUNT'S PRESENTATION. I JUST GOT THE FIVE-MINUTE SIT DOWN OR YOU'RE IN BIG TROUBLE SIGN FROM SCOTT. THIS WILL TAKE FIVE MINUTES OR LESS.

SO IN DECEMBER, AS YOU KNOW, WE ARE OVERSEEN BY THE STATE CONTROLLER WHO ANNUALLY CONVENES THE CITIZENS FINANCIAL ACCOUNTABILITY OVERSIGHT COMMITTEE TO TALK ABOUT SEVERAL THINGS. WE MET WITH MELIA COHEN, THE CONTROLLER, AND HER COMMITTEE IN SACRAMENTO IN HER OFFICES IN DECEMBER. AND WE TALKED -- THEY HAD A REVIEW BY MOSS-ADAMS OF OUR FINANCIAL AUDIT, WHICH, OF COURSE, WAS CRYSTAL CLEAR AS IT HAS BEEN FOR YEARS IN A ROW. SHOUT-OUT TO JEN LEWIS AND MEMBERS OF THE FINANCE TEAM FOR ANOTHER SPOTLESS AUDIT, WHICH IS EXACTLY HOW WE LIKE IT.

RAPHAEL PRESENTED ON THE LATEST CHANGES THAT WE HAVE MADE IN RESPONSE TO OUR PERFORMANCE AUDIT. FOR YOU NEW MEMBERS, WE HAVE TO DO A

PERFORMANCE AUDIT EVERY THREE YEARS TO CHECK ON HOW ALL OF THE THINGS THAT WE'RE DOING ARE BEST PRACTICES. AND THEY ALWAYS GIVE US A NUMBER OF THINGS TO IMPROVE ON, NOTABLY OVER TIME THAT GOES DOWN WITH EACH SUCCESSIVE AUDIT, WHICH IS THE WAY YOU LIKE IT. RAPHAEL DID A GREAT JOB IN PRESENTING IN RATHER SHORT TIME FRAME SHALL WE SAY.

AND THEN THE OTHER PART OF THE MEETING WAS I PRESENTED ON CIRM'S WORK AND ALL THE REALLY, REALLY INTERESTING THINGS THAT OUR FUNDED SCIENTISTS ARE DOING ACROSS THE STATE WITH UPDATES ON THE PORTFOLIO, EXAMPLES OF SPECIFIC PROJECTS, AND ANSWERING A LOT OF QUESTIONS THAT THEY HAD ON WHAT WE'RE DOING.

IN THE FIRST CFAOC MEETINGS, THERE WAS NONE OF THAT. IT WAS ALL THE FINANCIAL STUFF. ARE YOU BEING GOOD STEWARDS OF THE TAXPAYERS' DOLLARS? NOW THIS PART OF THE MEETING IS SOMETHING THEY REALLY LOOK FORWARD TO BECAUSE THEY'RE ALL FASCINATED BY WHAT THE CIRM FAMILY WRIT LARGE, INCLUDING ALL OF OUR WONDERFUL SCIENTISTS, ARE DOING. AND THEY WERE THE MOST ENGAGED THAT WE'VE EVER HAD THEM IN THIS MEETING. THEY ASKED A LOT OF QUESTIONS, GAVE US A CHANCE TO EXPAND ON WHAT WE'RE DOING. AND THEN THAT, I THINK, THAT WAS A VERY GOOD

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MEETING. IMPORTANTLY FOLLOWED AFTER THE MEETING, MARIA HAD A CHANCE TO SPEND TIME WITH THE CONTROLLER'S CHIEF OF STAFF AND DEPUTY AND HELPED DEVELOP THOSE RELATIONSHIPS FURTHER, WHICH ARE VERY CRITICAL BECAUSE WE WANT TO BE IN ABSOLUTELY BEST STANDING WITH ALL OF THE CONSTITUTIONAL OFFICERS. AND FOR THAT MATTER ALL THE LEGISLATIVE OFFICIALS IN SACRAMENTO TOO. SO MARIA GOT A LOT OF GOOD SINGLE ONE-ON-ONE TIME WITH THEM.

SO THAT WAS THE CFAOC MEETING, AND I THINK IT WAS GOOD. SO WITH THAT, UNLESS ANYBODY HAS ANY QUESTIONS, I MANAGED TO GET IN UNDER MR. TOCHER'S FIVE-MINUTE WARNING. AND THANK YOU ALL VERY MUCH.

(APPLAUSE.)

CHAIRMAN IMBASCIANI: J.T., THAT WAS A GREAT RECAP OF THE LECTURE. THANKS SO MUCH. WE COME NOW TO THE AGENDA ITEM 16, WHICH IS AN OPPORTUNITY FOR BOARD MEMBERS IF THEY HAVE ANY COMMENTS ON OUR APPLICATION REVIEW SUBCOMMITTEE PROCESS. ANY COMMENT FROM OUR ONLINE FOLKS? NO. OKAY.

GOING TO MOVE ON TO NO. 17, IT'S THE OPPORTUNITY FOR THE PUBLIC TO COMMENT ON ANYTHING THAT WAS NOT ON TODAY'S AGENDA.

VICE CHAIR BONNEVILLE: THERE IS ONE.

CHAIRMAN IMBASCIANI: PLEASE IDENTIFY.

MS. MANDAC: ANDREA, IF YOU COULD UNMUTE YOURSELF. ALL RIGHT. YOU'LL HAVE THREE MINUTES. YOUR TIME STARTS NOW.

MS. FERNANDEZ DE SOTO: THANK YOU SO MUCH FOR THE OPPORTUNITY. MY NAME IS ANDREA, AND I AM CALLING FROM EDMONTON, ALBERTA IN CANADA. I AM THE MOTHER OF JAKOB GUZIAK, THE EDMONTON BUBBLE BOY, AND WHO WAS EARLY DIAGNOSED WITH ADA-SCID THANKS TO THE NEWBORN SCREENING TEST. JAKOB IS THE CLOSEST EXAMPLE OF WHAT YOUR SUPPORT TO GENE THERAPIES FOR RARE CONDITIONS MEAN, THE SUPPORT YOU GIVE TO SCIENTISTS LIKE DR. DONALD KOHN WHO ARE READY CHANGE THE LIFE OF OUR LOVED ONES.

AN EXAMPLE OF THE TIRELESS FIGHT WE PARENTS HAVE TO DO IN ORDER TO ACCESS TREATMENT AROUND THE WORLD.

JAKOB COULD HAVE BEEN CURED IN THE EARLY YEARS OF HIS LIFE FOR AROUND $1.5 MILLION OR EVEN LESS IF GENE THERAPY WAS AVAILABLE HERE IN CANADA. FOUR AND A HALF YEARS IS THE TIME THAT WE WAITED TO SAVE OUR CHILD. THANKS TO YOUR SUPPORT AND THE COST OF HIS MEDICATION WE OBSERVED DURING THESE YEARS HAS BEEN AROUND A MILLION DOLLARS.

ONCE UPON A TIME, I WAS HERE PLEADING TO

ALL OF YOU TO GIVE A LIFE OPPORTUNITY TO MY SON AND TO ADA-SCID. TODAY I WANT TO GIVE TO ALL OF YOU MY INFINITE THANKS AT CIRM FOR YOUR BOLD DECISIONS FOR ADVOCATING FOR WHAT WAS RIGHT, FOR SUPPORTING DR. KOHN, AND MY HUMBLE PLEA TO KEEP IN YOUR CONSIDERATION THE MANY PARENTS THAT ARE HERE TODAY ASKING FOR THE SAME HELP I ONCE ASKED FOR MY CHILD.

I FIND MYSELF IN THE WORDS OF EACH MOM AND DAD HERE TODAY AND MY SON IN THEIR STORIES. PLEASE DO NOT FORGET THEM. DO NOT FORGET THEIR FIGHT, THEIR VOICE, AND WHAT YOUR ACTIONS MEAN TO ALL OF US. MORE THAN HOPE IS WHAT YOU GUYS HAVE GIVEN TO OUR FAMILY TODAY. PLEASE DO NOT STOP CHANGING THE LIVES AND GIVING THE SAME HOPE TO ALL THESE FAMILIES FOR A FUTURE OF COMMERCIALIZED GENE THERAPIES. THANK YOU.

CHAIRMAN IMBASCIANI: THANK YOU VERY MUCH. IT'S HARD TO FOLLOW THAT. OKAY. THANK YOU, BOARD MEMBERS, FOR YOUR ATTENDANCE AND YOUR PARTICIPATION IN TODAY'S MEETING.

I'M GOING TO ADJOURN THE MEETING SO THAT WE WOULD COME TOGETHER AGAIN IN SACRAMENTO AT 9 A.M. ON THURSDAY, 27 MARCH AT THE WESTIN HOTEL. THERE MIGHT BE TWO WESTINS IN SACRAMENTO. THIS ONE IS AT 4800 RIVERSIDE BOULEVARD. WE'RE PLANNING AND HOPING

FOR A DINNER, OUR USUAL CUSTOM, THE EVENING BEFORE. THE DETAILS OF THAT ARE TO BE DETERMINED. SO THAT'S THURSDAY, 27 MARCH. SEE YOU THEN. MEETING IS ADJOURNED. THANK YOU.

(THE MEETING WAS THEN CONCLUDED AT 4:07 P.M.)

REPORTER'S CERTIFICATE

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE PROCEEDINGS BEFORE THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE AND THE APPLICATION REVIEW SUBCOMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON JANUARY 30, 2025, WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

BETH C. DRAIN, CA CSR 7152
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