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: AS INDICATED ON THE AGENDA

DATE: JANUARY 19, 2017
10 A.M.

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

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I N D E X

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THURSDAY, JANUARY 19, 2017

10 A.M.

CHAIRMAN THOMAS: GOOD MORNING, EVERYBODY. WELCOME TO THE REGULAR MONTHLY MEETING OF THE ICOC AND THE APPLICATION REVIEW SUBCOMMITTEE. LIKE TO PROCEED WITHOUT FURTHER ADO. MARIA, WILL YOU PLEASE CALL THE ROLL.

MS. BONNEVILLE: I WILL. DAVID BRENNER. KEN BURTIS. DEBORAH DEAS. ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: HOWARD FEDEROFF. JUDY GASSON. SAM HAWGOOD. DAVID HIGGINS.

DR. HIGGINS: HERE.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: HERE.

MS. BONNEVILLE: SHERRY LANSING. KATHY LAPORTE. BERT LUBIN. SHLOMO MELMED. LAUREN MILLER. LLOYD MINOR. ADRIANA PADILLA. JOE PANETTA. FRANCISCO PRIETO.

DR. PRIETO: HERE.

MS. BONNEVILLE: CARMEN PULIAFITO. ROBERT QUINT.

DR. QUINT: HERE.

MS. BONNEVILLE: AL ROWLETT.

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MR. ROWLETT: HERE.
MS. BONNEVILLE: JEFF SHEEHY. OS STEWARD.
DR. STEWARD: HERE.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: HERE.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: HERE.
MS. BONNEVILLE: KRISTINA VUORI. DIANE WINOKUR.
MS. WINOKUR: HERE.
MS. BONNEVILLE: THANK YOU.
CHAIRMAN THOMAS: THANK YOU, EVERYBODY. AND A HAPPY NEW YEAR TO ALL.
WE'RE GOING TO PROCEED TO THE MAIN ITEM OF BUSINESS, WHICH IS CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO THE DISC2 PROGRAM ANNOUNCEMENT, WHICH IS PARTNERING OPPORTUNITIES FOR DISCOVERY STAGE RESEARCH PROJECTS, SO-CALLED QUEST AWARDS. I'M GOING TO TURN OVER FOR PRESENTATION NOW TO DR. GIL SAMBRANO.
DR. SAMBRANO: THANK YOU VERY MUCH, MR. CHAIRMAN. I'M GOING TO GO OVER AN OVERVIEW OF THE QUEST PROGRAM FOR WHICH WE ARE BRINGING APPLICATIONS FOR YOUR CONSIDERATION TODAY. I'M GOING TO GO THROUGH THE SLIDE DECK THAT WAS DISTRIBUTED. IF YOU

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HAVE IT ON HAND, THAT MIGHT BE HELPFUL. WE ARE ALSO GOING TO SHOW IT ON WEBEX. WE'RE HAVING LITTLE TECHNICAL CHALLENGES IN TERMS OF THE ORIENTATION OF IT, BUT I WILL TRY TO MAKE MY PRESENTATION AS SLIDE INDEPENDENT AS I CAN SO THAT YOU CAN FOLLOW ALONG REGARDLESS.

SO THE FIRST POINT I WANT TO MAKE AND WHAT IS SHOWN ON SLIDE 2 IS OUR FUNDING OPPORTUNITY THAT WE HAVE ESTABLISHED AT CIRM. THIS ONE, THE QUEST PROGRAM, FALLS UNDER THE DISCOVERY GROUP OF OPPORTUNITIES, AND IT IS OFFERED TWICE A YEAR, SO EVERY SIX MONTHS. AND GIVEN THE WAY WE'VE ADJUSTED THE CALENDAR FOR 2017, THE NEXT DEADLINE FOR THE QUEST PROGRAM IS COMING UP PRETTY SOON ON FEBRUARY 15TH. SO, AS ALWAYS, ANY OF OUR ONGOING OPPORTUNITIES, APPLICATIONS THAT ARE NOT FUNDED DURING THIS CYCLE ARE FREE TO APPLY TO THE NEXT CYCLE WHICH WILL COME FEBRUARY 15TH.

ON THE THIRD SLIDE, JUST ILLUSTRATING WHAT THE OBJECTIVE OF THE QUEST PROGRAM IS, WHICH IS TO PROMOTE DISCOVERY OF NEW STEM CELL-BASED TECHNOLOGIES THAT WILL BE READY FOR TRANSLATIONAL STUDY WITHIN TWO YEARS. SO THE GOAL HERE IS TO VERY QUICKLY TAKE WHAT WOULD BE PROOF OF CONCEPT STUDIES AND MAKE THEM READY FOR THE NEXT FUNDING OPPORTUNITY

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OF THE TRAN PROGRAM AT CIRM. AND THEN THESE PROJECTS, WE HOPE, WILL ULTIMATELY MAKE IT TO THE CLINIC AND IMPACT PATIENT CARE.

NOW, ON THE FOURTH SLIDE, I BEGIN TO DESCRIBE WHAT IT IS THAT QUALIFIES FOR THIS QUEST PROGRAM. AND THERE ARE DIFFERENT TYPES OF PROJECTS THAT CAN COME IN THAT PROPOSE A CANDIDATE THAT WOULD EITHER BE A THERAPEUTIC, A DIAGNOSTIC, A MEDICAL DEVICE, OR PERHAPS A TOOL.

AND THEN ON SLIDE 5, ANY OF THESE PRODUCT TYPES COULD INVOLVE A STEM OR PROGENITOR CELL THERAPY, A REPROGRAMMED CELL THAT IS ALSO A CELL THERAPY, A SMALL MOLECULE OR BIOLOGIC THAT STIMULATES, RECRUITS, OR TARGETS HUMAN ENDOGENOUS STEM CELLS OR CANCER STEM CELLS. IT COULD BE A DEVICE, DIAGNOSTIC, OR TOOL THAT IN SOME WAY USES A STEM OR PROGENITOR CELL, FOR EXAMPLE, TO DO SCREENING OF SMALL MOLECULE DRUGS OR OTHER, AND ONE THAT MIGHT ADDRESS A CRITICAL BOTTLENECK IN THE STEM CELL THERAPY FIELD; FOR EXAMPLE, HOW TO DELIVER A CELL THERAPY INTO A PARTICULAR AREA.

SO THE PROJECTS THAT WE ARE PRESENTING TO YOU THAT HAVE BEEN REVIEWED HAVE ALREADY GONE THROUGH AN ELIGIBILITY ASSESSMENT. SO WE'VE GONE THROUGH TO MAKE SURE THAT EVERYTHING THAT HAS COME

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IN FITS WITHIN THESE CRITERION. SO THEY ARE ALL ELIGIBLE. AND OUR INSTRUCTIONS TO REVIEWERS WHEN THEY REVIEWED THEM WAS THAT THESE ARE ELIGIBLE. AND SO THE FOCUS OF THE REVIEW FOR THE GWG FOCUSED ON FOUR MAIN CRITERIA, WHICH ARE SHOWN ON SLIDE 6.

AND THESE ARE DOES THE PROJECT HOLD THE NECESSARY SIGNIFICANCE AND POTENTIAL FOR IMPACT? THAT IS, DOES IT ALIGN WELL WITH THE QUEST PROGRAM ANNOUNCEMENT, AND DOES IT DELIVER VALUE, IF SUCCESSFUL, THAT COULD ULTIMATELY IMPACT PATIENTS.

SECOND, IS THE RATIONALE SOUND? THAT IS, IS THIS SOMETHING THAT MAKES SENSE, AND DOES THE PROPOSAL HAVE SUPPORTING DATA THAT SHOWS THAT THIS IS A GOOD APPROACH?

THIRD, IS THE PROJECT WELL-PLANNED AND DESIGNED?

AND FOURTH, IS THE PROJECT FEASIBLE? THAT IS, DO THEY HAVE THE RESOURCES AVAILABLE TO CONDUCT THE WORK? DO THEY HAVE A QUALIFIED TEAM? AND HAVE THEY SET OUT AN APPROPRIATE TIMELINE TO ACCOMPLISH THE WORK WITHIN THE EXPECTED TWO YEARS? SO THAT WAS THE FOCUS OF THE GWG WHEN THEY LOOKED AT THESE PROPOSALS.

ON THE NEXT SLIDE I'LL DESCRIBE THE SCORING SYSTEM THAT THE GWG THEN USES TO ASSESS THE

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MERIT OF THE APPLICATIONS BASED ON THOSE CRITERIA. WE HAVE A SCALE OF 1 TO 100 WITH A SCORE OF 85 TO 100 BEING A RECOMMENDATION TO FUND. AND WHEN ASSIGNING A SCORE OF 1 TO 84, IT'S NOT RECOMMENDED FOR FUNDING.

WE INSTRUCT REVIEWERS TO USE THE FULL SCALE FROM 1 TO 100 IN ORDER TO ASSESS MERIT. SO WHEN AN INDIVIDUAL REVIEWER ASSIGNS A SCORE, THEY'RE DOING A COUPLE OF THINGS. FIRST, THEY'RE DETERMINING IF AN APPLICATION SHOULD BE FUNDED OR NOT; THAT IS, THEY FIRST DETERMINE IS THIS SOMETHING I WANT TO SCORE WITHIN THE 85 TO 100 RANGE OR 1 TO 84. AND THEN THEY DETERMINE HOW FAR, HOW CLOSE TO THAT FUND LINE THE APPLICATION WOULD BE. AND WE DO ENCOURAGE THEM TO USE THE FULL SCALE BECAUSE IT IS IMPORTANT BOTH FOR YOU, THE BOARD MEMBERS, AS WELL AS THE PUBLIC AND THE APPLICANTS TO UNDERSTAND IF IT IS NOT RECOMMENDED FOR FUNDING, HOW FAR OFF WERE THEY. AND SO THE MORE INSTRUCTIVE THE SCORE CAN BE, THE BETTER IT IS FOR ALL.

SO ALL APPLICATIONS ARE SCORED BY THE SCIENTIFIC MEMBERS, AND WE USE, THEREFORE, THE MEDIAN OF ALL INDIVIDUAL GWG SCORES TO DETERMINE THE FINAL SCORE. AND THE MEDIAN IS WHAT PLACES THE APPLICATION IN THE FUND OR NOT FUND CATEGORY. THAT

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IS, IT'S THE VOTE. SO 50 PERCENT OR MORE OF THE GWG MEMBERS HAVE TO SCORE 85 OR ABOVE TO PLACE IT IN THAT CATEGORY. AND THEN WITHIN EACH OF THOSE CATEGORIES WE USE THE MEAN TO RANK THE APPLICATIONS WITHIN THAT CATEGORY.

SO FOR THE GWG RECOMMENDATIONS ON THIS PARTICULAR CYCLE OF QUEST, WE HAD, FOLLOWING THE DISCUSSION AND SCORING OF THESE APPLICATIONS, 14 THAT FELL INTO THE RECOMMENDED FOR FUNDING CATEGORY. AND THE OVERALL APPLICANT REQUEST, MEANING THE TOTAL DOLLAR AMOUNT THAT IS REQUESTED IN ALL OF THESE 14 APPLICATIONS, TOTALS TO $12.5 MILLION. HOWEVER, THE FUNDS THAT ARE AVAILABLE, BASED ON APPROVAL BY THE ICOC FOR THIS PARTICULAR CYCLE, IS ABOUT 21.4 MILLION. SO THERE IS A DEFICIT OF ABOUT 4 MILLION THAT WOULD PREVENT US FROM ACTUALLY FUNDING ALL 14. SO THAT IS IMPORTANT TO KNOW AS WE STEP INTO THIS.

THE NEXT SLIDE IS JUST A REMINDER THAT AT THE CONCLUSION OF EACH GWG REVIEW, WE HAVE ALL MEMBERS AND THE PATIENT ADVOCATE MEMBERS, WHO ARE ALSO REPRESENTATIVES ON THE BOARD, TO VOTE ON THE PROCESS THAT WAS DONE AS WELL; THAT IS, ON THE SCIENTIFIC RIGOR OF THE REVIEW. AND THE GWG VOTED UNANIMOUSLY THAT THE RIGOR AND THE FAIRNESS OF THE REVIEW WAS CONDUCTED IN AN APPROPRIATE MANNER.

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SO WHAT I'M GOING TO DO NEXT IS I'M GOING TO JUST BRIEFLY GO OVER EACH OF THE APPLICATIONS THAT ARE IN THE TOP TIER CATEGORY. I'M GOING TO TRY TO DO THIS AS BRIEFLY AS I CAN, BUT TRY TO PROVIDE TO YOU AS MUCH INFORMATION THAT MIGHT BE USEFUL JUST TO MAKE SURE THAT YOU UNDERSTAND AND KNOW WHAT EACH OF THESE APPLICATIONS IS ABOUT.

SO I'M GOING TO START WITH THE TOP ONE, JUST GOING IN RANK ORDER, WITHIN THAT CATEGORY. SO FOR APPLICATION 9526, WHICH IS ENTITLED, "GENE EDITING FOR FOXP3 IN HUMAN HSC'S," THIS IS AN APPLICATION THAT ADDRESSES WHAT'S CALLED THE IPEX SYNDROME. IT IS A RARE CHILDHOOD AND, UNFORTUNATELY, LETHAL AUTOIMMUNE SYNDROME THAT AFFECTS THE GUT, SKIN, GLANDS, AND IT'S CAUSED BY A MUTATION IN THE FOXP3 GENE IN T-CELLS.

SO THE APPROACH THAT THEY ARE TAKING IS A GENE-MODIFIED CELL THERAPY APPROACH, AND THEIR GOAL IS TO CONDUCT PROOF OF CONCEPT STUDIES TO TRANSLATE THIS FOXP3 GENE EDITING OF HSC'S TO THE NEXT STAGE.

THE APPLICATION GOT THE TOP SCORE OF 95 WITH A MEAN OF 93, AND 15 OUT OF THE 15 SCIENTIFIC MEMBERS SCORED IT IN THE RECOMMENDED FOR FUNDING CATEGORY.

SOME OF THE STRENGTHS AND WEAKNESSES THAT

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WERE IDENTIFIED: REVIEWERS THOUGHT THAT THIS WAS AN IDEAL CANDIDATE FOR A TARGETED GENE THERAPY APPROACH. THEY FOUND THAT THE APPROACH IS ONE THAT WOULD BE HIGHLY EFFICIENT AND ONE THAT WOULD HAVE A HIGH LIKELIHOOD TO WORK. AND IT PROVIDES POTENTIALLY A GOOD EXAMPLE OF WHAT MIGHT BE A CURATIVE POTENTIAL OF STEM CELLS. AND THE PRELIMINARY DATA, THEY FELT, WAS CONVINCING, AND IT WAS PUT TOGETHER BY A GREAT INVESTIGATIVE TEAM.

CONCERNS WERE RATHER MINOR. THERE WAS A COMMENT ABOUT THE RARITY OF THE DISEASE SO THAT THE IMPACT, THE BROAD IMPACT, MIGHT BE MUTED, ALTHOUGH IT WAS ALSO RECOGNIZED THAT THIS COULD BE APPLICABLE IN OTHER SETTINGS.

IN TERMS OF OTHER PROJECTS THAT CIRM IS FUNDING, THERE ARE NO CURRENTLY FUNDED PROJECTS THAT ARE SPECIFICALLY ADDRESSING IPEX SYNDROME.

THE NEXT APPLICATION IS 9649. THIS IS A TREATMENT FOR ZIKA VIRUS INFECTION AND NEUROPROTECTION EFFICACY. AND SO THIS IS TO TREAT ZIKA INFECTION, AND IT'S A SMALL MOLECULE THERAPEUTIC APPROACH. THE GOAL IS TO USE STEM CELLS HERE AS A TOOL BOTH TO STUDY AND TO VALIDATE A SMALL MOLECULE THAT THEY HAVE AS A POTENTIAL CANDIDATE TO DEVELOP AND TAKE TO THE CLINIC.

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THE APPLICATION RECEIVED A SCORE OF 93. THE MEAN WAS ALSO 93, AND 15 OUT OF THE 15 GWG SCIENTIFIC MEMBERS SCORED IT IN THE FUND CATEGORY.

SOME OF THE STRENGTHS AND WEAKNESSES: REVIEWERS THOUGHT THAT CERTAINLY THERE IS AN UNMET NEED AND A VERY IMPORTANT TOPIC THAT DOES NEED THERAPEUTIC INTERVENTION. THEY FELT THAT THE INVESTIGATOR IS VERY STRONG AS WELL AS THE PRELIMINARY DATA THAT WAS PRESENTED TO GO ALONG WITH THIS, AND THE EXPERIMENTAL PLAN THAT WAS LAID OUT WAS VERY WELL THOUGHT THROUGH.

THERE WERE SOME MINOR CONCERNS, THAT THE TESTING, FOR EXAMPLE, OF THIS PARTICULAR CANDIDATE HASN'T BEEN DONE IN THE PREGNANCY MODEL OF ZIKA THAT THE APPLICANT ESTABLISHED, AND THAT THEY MIGHT CONSIDER DOING THAT. AND THEN JUST A LACK OF CLARITY AS TO HOW THE SMALL MOLECULE DRUG ULTIMATELY WOULD BE DELIVERED INTO THE MOTHER OR FETUS. SO A LACK OF CLARITY THERE.

IN TERMS OF RELATED PROJECTS THAT WE MIGHT BE FUNDING, THAT WE ARE CURRENTLY NOT FUNDING ANYTHING THAT ADDRESSES THE ZIKA VIRUS INFECTION.

NEXT PROJECT IS 9565. THIS IS "PRECLINICAL DEVELOPMENT OF HUMAN HEPATOCYTE PROGENITOR CELLS FOR CELL THERAPY FOR LIVER

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DISEASE." AND CLEARLY THIS IS A CELL THERAPY. SO THEIR GOAL HERE IS TO DEVELOP A CANDIDATE HEPATOCYTE PROGENITOR THAT HAS ALL THE IDEAL FUNCTION AND CHARACTERISTICS THAT CAN BE USED TO TREAT LIVER DISEASE AND REPLACE HEPATOCYTES.

THIS APPLICATION RECEIVED A FINAL SCORE OF 90. THE MEAN WAS 91. AND, AGAIN, 15 OUT OF THE 15 SCIENTIFIC MEMBERS VOTED IN THE FUND CATEGORY.

STRENGTHS, THE APPLICATION ADDRESSES A CLEAR UNMET CLINICAL NEED. IT IS BASED ON A RECENT DISCOVERY OF HEPATOCYTE PROGENITOR CELLS IN THE MOUSE. AND SO THE GOAL IS TO TAKE THOSE STUDIES THAT SEEM VERY PROMISING IN THE MOUSE MODEL AND TRANSLATE IT TO HUMANS. AND REVIEWERS FELT THAT THE DESIGN OVERALL WAS VERY SOLID AND WOULD BE LIKELY TO PRODUCE MEANINGFUL RESULTS AND OVERALL HAD VERY GOOD PRELIMINARY DATA.

ONE CONCERN WAS THAT THE PRELIMINARY DATA ALSO INDICATES THAT THE TRANSPLANTATION OF THESE PROGENITOR CELLS MAY NOT PERSIST. SO IT IS ONE ISSUE THAT THE GROUP WILL NEED TO ADDRESS IF THEY WANT TO UTILIZE IT IN THE CLINIC ULTIMATELY.

AND THEY ALSO HAD THE RECOMMENDATIONS, SUCH AS TESTING THE RESTORATION OF LIVER FUNCTION IN AN IN-VIVO SETTING.

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IN TERMS OF PROJECTS THAT ARE SIMILAR, WE HAVE SEVEN DISCOVERY STAGE PROJECTS THAT TOTAL $12.7 MILLION INVESTMENT CURRENTLY FROM CIRM TO DEVELOP, TO STUDY LIVER DISEASE, NOT EXACTLY IDENTICAL TO THIS, BUT THAT ARE WITHIN THAT OVERALL PORTFOLIO AREA.

NEXT APPLICATION IS 9615. THIS IS ENTITLED "TARGETED OFF-THE-SHELF IMMUNOTHERAPY TO TREAT REFRACTORY CANCERS." SO THE INDICATION IS FOR BOTH SOLID AND HEMATOLOGIC CANCERS. IT IS A GENE-MODIFIED CELL IMMUNOTHERAPY APPROACH, AND THEIR GOAL HERE IS TO DEVELOP PROOF OF CONCEPT DATA IN CANDIDATES FOR BOTH SOLID TUMORS USING NATURAL KILLER CELLS THAT ARE MODIFIED, AND THEN IN PARALLEL A NONMODIFIED NATURAL KILLER CELL APPROACH FOR TREATING LEUKEMIA, SUCH AS AML.

THE APPLICATION RECEIVED A SCORE OF 90. THE MEAN WAS 91. AND, HERE AGAIN, 15 OUT OF THE 15 GWG MEMBERS PLACED IT IN THE FUND CATEGORY.

IN TERMS OF STRENGTHS AND WEAKNESSES THAT WERE HIGHLIGHTED, REVIEWERS APPRECIATED AND LIKED THE IDEA THAT THIS COULD LEAD TO A UNIVERSAL OR OFF-THE-SHELF CELL THERAPY TO TREAT MANY PATIENTS, AND THE IMPACT COULD BE BROAD. THE TARGETS THAT ARE BEING STUDIED ARE RELEVANT. IN THIS CASE, THEY ARE

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STUDYING OVARIAN CANCER AND LEUKEMIA. THEY HAVE A VERY PURE NATURAL KILLER CELL POPULATION THAT THEY CAN ACHIEVE, AND THEY HAVE GOOD PRELIMINARY DATA TO DEMONSTRATE FEASIBILITY.

SOME CONCERNS WERE THAT THE STUDIES EXPLORE MANY COMBINATIONS OF THE SINGLE TRANSDUCTION ELEMENTS THAT MIGHT END UP BEING A BIT AMBITIOUS FOR THE TWO-YEAR TIMELINE.

AND THEN IN TERMS OF SIMILAR PROJECTS THAT ARE IN THE CANCER PORTFOLIO, CLEARLY WE HAVE SEVERAL, THERAPEUTIC AND OTHER APPROACHES FOR CANCER. THERE ARE FIVE IN THE CLINICAL PROGRAM THAT TOTAL 51.1 MILLION, TWO IN THE TRANSLATIONAL PROGRAM OF 9.9 MILLION, AND SEVEN IN THE DISCOVERY REALM FOR 13.1 MILLION.

THE NEXT APPLICATION IS 9569 TITLED "HNSC-MEDIATED DELIVERY OF APICCT1 AS A CANDIDATE THERAPEUTIC FOR HUNTINGTON'S DISEASE." SO THIS IS A THERAPY OF GENE MODIFIED CELLS AS WELL AS A BIOLOGIC FOR TREATING HUNTINGTON'S DISEASE. IT IS KIND OF A DUAL APPROACH THAT UTILIZES THE NEURAL STEM CELLS AS ONE ASPECT OF TREATING THE DISEASE AS WELL AS THE CELLS BEING ABLE TO SECRETE THE APICCT1 PROTEIN FOR ADDITIONAL BENEFIT.

THE GOAL OF THESE STUDIES IS TO DO A PROOF

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OF CONCEPT TO ESTABLISH A CANDIDATE FOR TRANSLATION OF THIS DUAL APPROACH.

THE FINAL SCORE FOR THIS APPLICATION WAS A 90. THE MEAN WAS ALSO A 90. HERE WE HAVE 13 MEMBERS SCORING THE APPLICATION IN THE TOP TIER AND ONE MEMBER SCORING IT IN THE DO NOT FUND CATEGORY.

CLEARLY, THIS APPLICATION ADDRESSES AN UNMET NEED, AND THIS IS RECOGNIZED BY REVIEWERS. THEY FELT THAT THE PRELIMINARY DATA IS STRONG AND WARRANTS FURTHER PRECLINICAL WORK, AND FOUND IT TO BE A CLEVER STRATEGY FOR ENHANCING THE EFFICACY BY DOING THE COMBINATION OF THESE NSC WITH THE API DELIVERY.

THERE WERE SEVERAL CONCERNS RAISED. SOME, THAT OUT OF SEVEN TRIALS WITH STEM CELLS, ONLY ONE HAS SHOWN SOME MARGINAL GAIN, WHICH WAS SHORTLIVED. AND THE PERSISTENCE OF THE CELLS, ONCE INTRODUCED, MAY BE A HURDLE FOR DEVELOPMENT, AS WELL AS CELL SURVIVAL ONCE THEY ARE TRANSPLANTED. ALSO, IT IS UNCLEAR HOW THE DELIVERY OF THE APICCT1 MAY BE SUSTAINED ULTIMATELY, AND SOME CONCERNS OF OFF-TARGET EFFECTS OF THE APICCT1. AND THERE ARE OTHER MINOR CONCERNS AS WELL.

IN TERMS OF PROJECTS THAT ARE WITHIN THE SAME PORTFOLIO AREA, THERE IS ONE TRAN STAGE PROJECT

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AT 5 MILLION.

THE NEXT APPLICATION IS 9624. THIS IS ENTITLED "PROTEIN TYROSINE PHOSPHATASE-SIGMA INHIBITORS FOR HEMATOPOIETIC REGENERATION." THE INDICATION HERE IS FOR SITUATIONS SUCH AS MYELO-ABLATION OR OTHER CONDITIONS WHERE PATIENTS NEED IMMUNE AND/OR BLOOD REGENERATION. IT IS A SMALL MOLECULE APPROACH. AND THE GOAL HERE IS TO SCREEN AND STUDY SEVERAL PEAK SIGMA INHIBITORS TO IDENTIFY AND CHARACTERIZE AN IDEAL CANDIDATE THAT THEY CAN TAKE FOR TRANSLATION.

THIS APPLICATION RECEIVED A SCORE OF 90. THE MEAN WAS ALSO 90. AND WE HAD 14 OUT OF 14 MEMBERS SCORE THIS APPLICATION WITHIN THE FUND CATEGORY.

SOME OF THE STRENGTHS AND CONCERNS: THERE IS A NEED FOR A DRUG THAT CAN STIMULATE HEMATOPOIETIC STEM CELLS, SO IT WOULD BE A PROPOSAL THAT, IF SUCCESSFUL, WOULD HAVE GREAT IMPACT. THEY FELT THAT MOLECULE OPTIMIZATION, CHEMICAL MODIFICATIONS PROPOSED ARE VERY STRONG, AND THAT USE OF HUMAN STEM CELLS IS A STRENGTH FOR THIS PARTICULAR PROPOSAL.

SOME MINOR CONCERNS IN TERMS OF INTERPRETING THE DATA ON ONE OF THE FIGURES AND BOTH

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COMMENTS RELATE TO THAT.

IN TERMS OF RELATED PROJECTS, THERE ARE TWO CLINICAL PROJECTS THAT TOTAL 19.1 MILLION AND ONE DISCOVERY STAGE PROJECT FOR 5.2 MILLION IN THIS AREA.

NEXT APPLICATION IS 9596 ENTITLED "DIRECT CARDIAC REPROGRAMMING FOR REGENERATIVE MEDICINE." THIS IS FOR HEART FAILURE AND IS A GENE THERAPY APPROACH. THE GOAL HERE IS TO CONDUCT PROOF OF CONCEPT STUDIES FOR TESTING AN APPROACH WHICH BASICALLY REPROGRAMS CELLS WITHIN THE HEART IN ORDER TO REPOPULATE CARDIAC MYOCYTES IN THE HEART TO REPAIR THE TISSUE. AND WHAT THEY ARE TESTING IS THE ABILITY TO DELIVER GENES AND ALSO THE USE OF A SMALL MOLECULE TO ENHANCE THE REPROGRAMMING OF THE CELLS IN THE HEART. AND ALL OF THIS TO ESTABLISH A CANDIDATE THAT THEY CAN THEN TRANSLATE ONTO THE NEXT STAGE.

THIS APPLICATION RECEIVED A SCORE OF 88. THE MEAN WAS ALSO 88. AND THE GWG, 14 OUT OF 14 MEMBERS SCORED IT IN THE FUND CATEGORY.

REVIEWERS FELT THAT THIS APPLICATION HAD A STRONG RATIONALE, AS WELL AS STRONG PRELIMINARY DATA, AND A GOOD OVERALL DESIGN.

SOME OF THE CONCERNS RELATE TO OFF-TARGET

EFFECTS THAT THE VECTORS MAY HAVE, AND THEIR EFFECT ON THE EXISTING CARDIOMYOCYTES WERE NOT DISCUSSED SUFFICIENTLY.

THERE ARE OTHER RELATED AWARDS; THAT IS, THOSE THAT ARE IN THE FIELD OF HEART FAILURE. THERE ARE THREE CLIN STAGE PROJECTS THAT TOTAL 42.2 MILLION AND 11 DISCOVERY STAGE PROJECTS AT 19.4 MILLION.

THE NEXT APPLICATION IS 9635 ENTITLED "DESIGNING A CELLULAR NICHE FOR TRANSPLANTATION OF HUMAN EMBRYONIC STEM CELL-DERIVED BETA CELLS." THIS IS FOR TYPE 1 DIABETES, AND IT IS CELL THERAPY APPROACH. THE GOAL HERE TO CONDUCT PROOF OF CONCEPT STUDIES TO DEVELOP WHAT WOULD BE AN ISLET CELL GROUP OF CELLS SO THEY CREATE A COMPOSITE, NOT ONLY BETA CELLS, BUT OTHER CELL TYPES THAT CAN THEN BE TRANSPLANTED OR POTENTIALLY PLACED IN AN ENCAPSULATION DEVICE TO TREAT DIABETES.

THE FINAL SCORE FOR THIS APPLICATION IS AN 88, MEAN WAS ALSO AN 88. THERE WERE 15 OUT OF 15 GWG REVIEWERS THAT SCORED IT IN THE FUND CATEGORY.

REVIEWERS FELT THAT THE PROPOSAL WAS BROUGHT BY A PROMISING RISING STAR INVESTIGATOR THAT HAS OUTSTANDING PRELIMINARY OUTCOMES. AND THEY WERE OVERALL IMPRESSED BY THE DATA THAT THEY HAVE SO FAR.

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IN TERMS OF CONCERNS, THERE WERE SEVERAL, BUT I THINK OVERALL PERHAPS MINOR. THEY FELT THAT THE PROPOSAL WAS OVER AMBITIOUS, SO THIS COULD CERTAINLY TAKE IT BEYOND THE TWO YEARS. SO THAT WAS ONE OF THE MAJOR CONCERNS. LACK OF DISCUSSION ABOUT CERTAIN KEY ELEMENTS, SUCH AS THE NEED FOR IMMUNOISOLATION DEVICES OR IMMUNOSUPPRESSION, AND WHETHER THE SORT OF ISLET NICHE CELLS FROM CADAVERIC PANCREATIC ISLETS WOULD BE NECESSARY OR ULTIMATELY UTILIZED IN THE FINAL PRODUCT OR NOT COULD POSE A PROBLEM IN TERMS OF THE PURPOSES OF USING STEM CELLS FROM A SOURCE SUCH AS HESC'S.

THERE ARE A FEW PROJECTS IN THE AREA OF DIABETES. THERE ARE CURRENTLY THREE CLINICAL STAGE PROJECTS THAT WE FUND FOR A TOTAL OF $30.2 MILLION AND ONE AT THE TRANSLATION STAGE FOR 5 MILLION.

THE NEXT APPLICATION ALSO IN THE AREA OF TYPE 1 DIABETES. THIS IS 9559 ENTITLED "THIN FILM ENCAPSULATION DEVICES FOR HUMAN STEM CELL-DERIVED INSULIN-PRODUCING CELLS." IT IS INTENDED FOR CELL THERAPY, BUT IT'S THE DEVELOPMENT OF AN ENCAPSULATION DEVICE. SO THEIR GOAL IS TO TEST FEATURES AND ENHANCEMENT OF THE ENCAPSULATION DEVICE TO SHOW FUNCTION AND PROOF OF CONCEPT FOR SUBSEQUENT TRANSLATION OF THIS PRODUCT.

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THE FINAL SCORE FOR THIS APPLICATION IS IN AN 87. THE MEAN IS ALSO AN 87. HERE THERE WERE 13 GWG MEMBERS THAT SCORED IT WITHIN THE FUND CATEGORY AND TWO THAT SCORED IT IN THE DO NOT FUND CATEGORY.

THERE WERE SEVERAL STRENGTHS HIGHLIGHTED, SUCH AS THE RATIONALE WAS FELT TO BE STRONG. THEY APPRECIATED THE PARTICIPATION OF THREE CO-PI'S WITH EXPERTISE THAT ALL CONTRIBUTE TO DEVELOPING THIS PROJECT. THE DESIGN OF THE DEVICE AND APPROACH WAS NOVEL, AND THEY FELT THAT THE USE OF THREE MODELS ADDED SCIENTIFIC RIGOR TO OVERALL DESIGN.

THERE WERE SOME CONCERNS, SOME MINOR WEAKNESSES ON THE APPROACH FOR OPTIMIZING THE DEVICE, FUNCTIONAL DATA SHOWING THAT INSULIN-SECRETING ISLETS IN THE DEVICES CAN REVERSE DIABETES. SO ADEQUATE PRELIMINARY DATA SUPPORTING THE IDEA THAT ULTIMATELY THESE DEVICES CAN SUPPORT CELLS AND WOULD ALLOW THEM TO SENSE GLUCOSE AND FUNCTION AS ONE WOULD LIKE.

THERE ARE, AGAIN, THE SAME PROJECTS IN THE AREA OF DIABETES, THREE IN THE CLINICAL AREA AND ONE IN THE TRANSLATION PROJECT. SAME AS THE PREVIOUS.

THE NEXT APPLICATION IS 9610. THIS ONE IS ENTITLED "CRISPR/DCAS9 MUTANT TARGETING SNCA PROMOTER FOR DOWNREGULATION OF ALPHA-SYNUCLEIN

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EXPRESSION AS A NOVEL THERAPEUTIC APPROACH FOR PARKINSON'S DISEASE." SO OBVIOUSLY THIS IS TARGETED TO PARKINSON'S DISEASE, AND IT IS A GENE THERAPY. HERE, THE GOAL IS TO USE HUMAN STEM CELL-DERIVED NEUROPROGENITOR CELLS TO TEST THE GENE THERAPY APPROACH AND ESTABLISH A PROOF OF CONCEPT FOR TRANSLATION.

THE APPLICATION RECEIVED A FINAL SCORE OF 85. THE MEAN WAS 87. THERE WERE 11 GWG REVIEWERS THAT SCORED IT IN THE FUND CATEGORY AND FOUR THAT SCORED IT IN THE DO NOT FUND.

REVIEWERS FELT THAT THERE WAS SUBSTANTIAL PRELIMINARY DATA THAT INDICATES THE STRATEGY COULD BE EFFECTIVE IN KNOCKING DOWN THE SNCA LEVELS. IT IS A DEVICE PROPOSAL, MEANING THIS IS AN APPLICATION THAT IS COMING TO THIS PANEL FOR THE SECOND TIME, AND THEY FELT THAT THE APPLICANT AMPLY ADDRESSED MANY OF THE CONCERNS FROM THE PREVIOUS REVIEW AND THAT THEY HAVE OUTLINED A CLEAR PLAN FOR MOVING FORWARD.

THERE WERE CONCERNS THAT ULTIMATELY THE HYPOTHESIS THAT THE ALPHA-SYNUCLEIN REGULATION IN THE SPORADIC PARKINSON'S DISEASE MAY NOT BE RELEVANT. SO IF THE HYPOTHESIS IS WRONG, THEN THE VALUE OF THE PRODUCT MAY BE LIMITED. THERE WAS

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CONCERN ABOUT OFF-TARGET EFFECTS, SUCH AS WHETHER THE GENE THERAPY VIRUS WOULD BE TAKEN UP BY OTHER CELLS AND WHAT THE CONSEQUENCES WOULD BE. ALSO, THAT ONLY A SMALL AMOUNT OF THE ALPHA-SYNUCLEIN IS SUFFICIENT TO CREATE AN ABNORMAL PROCESSING, AND SO UNCLEAR HOW MUCH KNOCKDOWN WILL BE NECESSARY TO CHANGE THE COURSE OF THE DISEASE, BUT OBVIOUSLY SOMETHING THAT THE APPLICANT CAN AND WOULD NEED TO ADDRESS.

IN TERMS OF OTHER PROJECTS IN THIS AREA, WE HAVE AND ARE SUPPORTING THREE DISCOVERY STAGE PROJECTS IN THE AREA OF PARKINSON'S FOR 4.8 MILLION.

THE NEXT APPLICATION IS 9631 ENTITLED "IDENTIFICATION AND CHARACTERIZATION OF THE OPTIMAL HUMAN NEURAL STEM CELL LINE FOR THE TREATMENT OF TRAUMATIC BRAIN INJURY." SO THIS IS A CELL THERAPY APPROACH, AND THE GOAL OF THIS PROPOSAL IS TO TEST SEVERAL CELL LINES TO IDENTIFY AN IDEAL CANDIDATE THAT THEY CAN TAKE FORWARD TO TRANSLATION.

THIS RECEIVED A FINAL SCORE OF 85 WITH A MEAN OF 87. NINE OF THE GWG REVIEWERS SCORED IT WITHIN THE FUND CATEGORY, FIVE SCORED IT IN THE DO NOT FUND CATEGORY.

THE REVIEWERS NOTED THAT TRAUMATIC BRAIN INJURY OR TBI IS A MAJOR UNMET NEED. SOME OF THE

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STRENGTHS NOTED WAS THAT THIS APPROACH OF SMALL REDUCTION IN LESION VOLUME COULD HAVE A SIGNIFICANT LONG-TERM BENEFIT FOR PATIENTS, AND THEY FELT THAT THE WORK AS PROPOSED WAS VERY CAREFUL WORK ALTHOUGH MIGHT BE INCREMENTAL. BUT THEY FELT THAT THE PROPOSAL HAS THE RIGHT FOCUS AND ATTENTION TO DETAIL THAT WOULD BE NECESSARY TO MAKE IT SUCCESSFUL. SO ALSO VERY LOGICAL, AND THE PI HAS A GREAT RECORD OF PERFORMANCE ON CIRM GRANTS.

SOME OF THE CONCERNS RELATE TO WHETHER THE APPROACH MIGHT MAKE SENSE, THAT IS, LOCAL INJECTION FOR WHAT IS A MULTIFOCAL DISEASE, MAY BE A PROBLEM AS IT MAY NOT BE APPLICABLE TO THIS PATIENT POPULATION IF ULTIMATELY MANY INJECTIONS WOULD BE REQUIRED. THE NEED FOR IMMUNOSUPPRESSION WAS A CONCERN FOR SOME REVIEWERS AND COULD POSE A BARRIER TO TRANSLATION. SEVERAL FELT THE PRELIMINARY DATA TO SOME DID NOT SEEM TO BE SUFFICIENTLY ROBUST OR COMPELLING.

AND IN TERMS OF PROJECTS IN THIS AREA, WE ARE CURRENTLY NOT FUNDING ANYTHING IN THE TRAUMATIC BRAIN INJURY ARENA.

THREE MORE. 9542 IS THE NEXT ONE. THIS ONE IS ENTITLED "MULTIPOTENT CARDIOVASCULAR PROGENITOR REGENERATION OF THE MYOCARDIUM AFTER MI."

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SO THIS IS ANOTHER APPLICATION IN THE AREA OF HEART FAILURE. IT IS A CELL THERAPY APPROACH. AND THE GOAL IS TO CONDUCT PROOF OF CONCEPT STUDIES IN ANIMAL MODELS IN ORDER TO ESTABLISH AN IDEAL CANDIDATE THAT THEY CAN TAKE FOR TRANSLATION.

THIS APPLICATION RECEIVED A SCORE OF 85. THE MEAN WAS ALSO 85. HERE, THERE WERE NINE GWG REVIEWERS THAT SCORED IT WITHIN THE FUND CATEGORY AND FIVE THAT SCORED IT IN THE DO NOT FUND CATEGORY.

SOME OF THE REVIEWER COMMENTS WERE THAT SOME FOUND THAT THE SCIENTIFIC BACKGROUND WAS SOLID, THE IDENTIFICATION OF WHAT APPEARS TO BE A TRUE CARDIAC PROGENITOR IS BOTH NOVEL AND A STRENGTH AND PERHAPS ADVANTAGE TO THIS PROJECT. THEY LIKED IN GENERAL THE PRELIMINARY DATA, AND THE WAY OF DELIVERING AND TARGETING THE CELLS WAS THOUGHT TO BE INNOVATIVE.

SOME CONCERNS, IT WAS NOT CLEAR TO SOME IF THE PROPOSED CELL TYPE HAS A HIGH OR HIGHER LIKELIHOOD OF BEING SUCCESSFUL OVER OTHERS. THAT IS, THEY WERE PERHAPS LOOKING FOR ADDITIONAL JUSTIFICATION FOR WHY THESE CELLS WOULD INTEGRATE IN THE HEART AND PERSIST ADEQUATELY TO FUNCTION AS THEY WOULD HOPE.

WITHIN THE AREA OF HEART FAILURE, WE HAVE

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THREE CLINICAL PROJECTS THAT TOTAL 42.2 MILLION AND 11 DISCOVERY STAGE PROJECTS AT 19.4 MILLION.

THE NEXT PROPOSAL IS 9637 ENTITLED "GENOME EDITING TO CORRECT CYSTIC FIBROSIS MUTATIONS IN AIRWAY STEM CELLS." THIS IS A GENE-MODIFIED CELL THERAPY APPROACH TO TREAT CYSTIC FIBROSIS, AND THE GOAL IS TO DEVELOP AND TEST TWO GENE CORRECTION APPROACHES TO TREAT THE CYSTIC FIBROSIS MUTATION; THAT IS, THE CFTR GENE.

THIS APPLICATION RECEIVED A SCORE OF 85. THE MEAN WAS 85. THERE WERE TEN GWG REVIEWERS THAT SCORED IT WITHIN THE FUND CATEGORY AND FIVE THAT SCORED IT IN THE DO NOT FUND CATEGORY.

REVIEWERS, SOME OF THE COMMENTS ARE THAT THEY FOUND THE SCIENTIFIC RATIONALE TO BE SOUND. SIGNIFICANCE AND NOVELTY AND THE EXPERTISE OF THE TEAM WERE STRENGTHS OF THE PROPOSAL. CLEARLY AN UNMET NEED. IT IS AN AMBITIOUS PROPOSAL PERHAPS, BUT HAS A HIGH POTENTIAL FOR DELIVERING VALUE.

SOME OF THE CONCERNS ARE THAT CORRECTION OF THE CFTR GENE IS A CHALLENGE, PARTICULARLY IN THE TYPES OF CELLS AND SETTING THAT THEY ARE ATTEMPTING HERE, AND IT HASN'T YET BEEN ACHIEVED. SO IT IS SOMETHING THAT MAY PROVE TO BE DIFFICULT. A METHOD FOR PURIFYING OR ENRICHING THE SUCCESSFULLY

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GENE-CORRECTED CELLS AT LEAST WAS FOUND TO BE A LIMITATION BY SOME REVIEWERS. INSUFFICIENT FOCUS ON THE CELL TYPES THAT ARE SUCCESSFULLY GENE CORRECTED ADDRESSES THE SAME CONCERN. AND, AGAIN, ENGRAFTMENT OF THE CELLS INTO THE AIRWAY EPITHELIUM WAS THOUGHT TO BE NOT A TRIVIAL UNDERTAKING. AND SO THIS COULD EASILY TAKE THEM BEYOND THE TWO-YEAR TIMELINE TO ACHIEVE. THERE WAS JUST SOME LACK OF CLARITY ON WHY THE APPLICANT IS PURSUING TWO APPROACHES, ONE THAT IS FOCUSED ON A VERY SPECIFIC MUTATION AND ONE THAT IS KIND OF BROADER ACROSS DIFFERENT ONES. THEY FELT IF THEY'RE ESTABLISHING A UNIVERSAL STRATEGY, WHY NOT USE JUST THE ONE FOR ALL CASES. SO THAT'S JUST A LACK OF DISCUSSING THE RATIONALE BEHIND THAT. AND THEN ALSO A SUGGESTION THAT THE IMPLANT SITE THAT WAS PROPOSED, THAT IS, IN THE NASAL PASSAGE, MAY NOT BE AS INFORMATIVE AS PLACEMENT INTO THE LUNG.

IN TERMS OF RELATED PROJECTS, WE'RE NOT CURRENTLY FUNDING ANYTHING IN THE AREA OF CYSTIC FIBROSIS.

THE LAST APPLICATION IN THE FUND CATEGORY, 9460, ENTITLED "MICROENVIRONMENT FOR HUMAN INDUCED PLURIPOTENT STEM CELL-DERIVED PACEMAKING CARDIOMYOCYTES." IT IS A CELL THERAPY APPROACH TO TREAT CARDIAC ARRHYTHMIA. AND THE GOAL OF THIS

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PROJECT IS TO IDENTIFY AN IDEAL PACEMAKER CARDIOMYOCYTE THAT COULD BE USED TO BE TRANSLATED TO POTENTIALLY DEVELOP A BIOLOGIC PACEMAKER.

THE FINAL SCORE FOR THIS ONE IS AN 85. THE MEAN IS AN 80. THERE WERE NINE GWG REVIEWERS THAT SCORED IT IN THE FUND CATEGORY AND SIX THAT SCORED IT IN THE DO NOT FUND CATEGORY.

SOME OF THE STRENGTHS AND CONCERNS THAT WERE HIGHLIGHTED WAS THAT THIS IS A WELL-FOCUSED, WELL-WRITTEN PROPOSAL. IT HAS A NOVELTY IN SEVERAL ASPECTS IN TERMS OF PARTICULARLY USING EXTRACELLULAR MATRIX TO DRIVE THE DIFFERENTIATION AND MAINTENANCE OF THE CELLS THAT THEY ARE GENERATING TO HAVE A STRONG PRELIMINARY DATA FOR THIS.

SOME OF THE CONCERNS RELATED TO WHETHER THERE IS A NEED FOR A BIOLOGICAL ALTERNATIVE TO DEVICE PACEMAKERS. THERE WAS DISCUSSION THAT THERE ARE MANY NEW ADVANCES IN WIRELESS PACEMAKERS AND BIOSENSORS THAT ARE IMPROVING THE FIELD, AND THE PACEMAKER DESIGN MAY OVERCOME SOME OF THE CURRENT ISSUES, AND THE OVERALL FEASIBILITY OF THIS APPROACH FOR SOME SEEMED LOW. AND SCIENTIFICALLY THEY FOUND IT TO BE QUITE INTERESTING, BUT MIGHT REQUIRE MORE THOUGHT IN TERMS OF HOW TO ULTIMATELY GET IT TO PATIENTS.

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IN TERMS OF OTHER PROJECTS THAT ARE IN THIS PORTFOLIO AREA, TWO DISCOVERY STAGE PROJECTS FOR A TOTAL OF 7.7 MILLION IN THIS AREA.

SO THAT CONCLUDES A SUMMARY OF THE PROJECTS THAT ARE IN THE FUND CATEGORY.

CHAIRMAN THOMAS: THANK YOU, DR. SAMBRANO. BEFORE TURNING THE PROGRAMMATIC PORTION OF OUR REVIEW OVER TO DR. PRIETO, I WANTED TO MAKE A COMMENT ABOUT THE PUBLIC COMMENT COMPONENT OF TODAY'S MEETING. IN THE PAST WE'VE HAD INSTANCES WHERE THERE HAVE BEEN PROPOSALS THAT HAVE ACTUALLY, FOR ONE REASON OR ANOTHER, NEVER COME UP FOR CONSIDERATION WITH THE BOARD AT A GIVEN MEETING AND HAVE HAD PUBLIC COMMENTERS WHO HAVE NOT BEEN ABLE TO PRESENT THEIR THOUGHTS IN ADVANCE OF ANY VOTES ON THE LIST OF PROJECTS UNDER CONSIDERATION.

WE'VE DETERMINED THAT THAT IS NOT A GOOD THING. AND SO WE ARE FOR THIS MEETING CHANGING THE PUBLIC COMMENT TIMING TO ADDRESS THAT ISSUE. SO FOR THIS MEETING WE'RE GOING TO HAVE ALL PUBLIC COMMENT PRECEDE ANY DISCUSSION ON ANY OF THE INDIVIDUAL PROPOSALS. SO I WOULD ENCOURAGE ALL THOSE EITHER IN ATTENDANCE HERE OR AT OTHER SITES ON THE PHONE WHO DO WISH TO GIVE PUBLIC COMMENT TO BE PREPARED TO GIVE THAT COMMENT RIGHT AFTER I STOP TALKING HERE.

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WHEN WE GET TO INDIVIDUAL PROJECTS, BECAUSE WE WILL HAVE HAD PUBLIC COMMENT AT THE OUTSET, THERE WILL NOT BE PUBLIC COMMENT FOR EACH OF THE INDIVIDUAL PROJECTS BEING CONSIDERED. OF COURSE, IF THERE ARE MEMBERS OF THE BOARD THAT DO HAVE QUESTIONS WITH RESPECT TO INDIVIDUAL PROJECTS WHO WISH TO ASK THOSE QUESTIONS AND THERE HAPPENS TO BE A MEMBER OF THE PUBLIC HERE WHO CAN ADDRESS ANY SUCH QUESTIONS, THAT WILL, OF COURSE, BE THE ORDER OF THE DAY. BUT SHORT OF ANY QUESTIONS THAT ANY INDIVIDUAL BOARD MEMBERS HAVE, WE WILL NOT BE HAVING PUBLIC COMMENT DURING THE CONSIDERATION OF ANY OF THE INDIVIDUAL PROJECTS.

MS. BONNEVILLE: QUESTION. ARE THERE ANY MEMBERS OF THE PUBLIC AT THE OTHER LOCATIONS? WE HAVE SEVERAL HERE, AND I JUST WANTED TO CHECK IN. NO OTHER MEMBERS OF THE PUBLIC?

CHAIRMAN THOMAS: THANK YOU. SENATOR TORRES.

MR. TORRES: I JUST WANTED TO MAKE SURE THAT WHOEVER IS LISTENING THAT SEVEN OF US, AS PATIENT ADVOCATES, THIS IS THE SECOND TIME WE'RE REVIEWING THESE APPLICATIONS. SO THAT THE PEOPLE REALIZE THIS IS NOT JUST A PRO FORMA REVIEW BY THE BOARD AB ANITIO. RATHER, SOME OF US WHO ARE BOARD

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MEMBERS AND PATIENT ADVOCATES HAVE BEEN PART OF THIS PROCESS BEFORE THIS BOARD MEETING.

CHAIRMAN THOMAS: THANK YOU. VERY IMPORTANT POINT, SENATOR TORRES.

OKAY. WITH THAT AS THE GROUNDRULE, NOW GOING TO --

MS. BONNEVILLE: ARE WE GOING TO DO IT IN ORDER?

CHAIRMAN THOMAS: YES. HERE AT CIRM HEADQUARTERS WE HAVE A SIGN-IN SHEET FOR PUBLIC COMMENT. LOOKS LIKE WE HAVE SEVEN FOLKS GOING TO SPEAK. WE'RE GOING TO PROCEED IN ORDER, AND I WOULD ADVISE EVERYBODY YOU HAVE A CAP OF THREE MINUTES FOR YOUR PUBLIC COMMENT. SO PLEASE GIVE YOUR NAME AND YOUR INSTITUTION THAT YOU ARE AFFILIATED WITH.

DR. JUELSGAARD: COULD I JUST MAKE ONE QUICK COMMENT ABOUT GIL'S PRESENTATION BEFORE WE DO THIS?

CHAIRMAN THOMAS: CERTAINLY.

DR. JUELSGAARD: I JUST WANT TO THANK DR. SAMBRANO. I THINK THAT WAS, ALTHOUGH LENGTHY, AN EXCELLENT PRESENTATION. AND I PARTICULARLY APPLAUD THE STAFF FOR ADDING THIS TIME THE OTHER PROJECTS THAT WE HAVE GOING ON IN THE AREA AND THE AMOUNT OF FUNDING ASSOCIATED WITH THEM. WE HAVEN'T ADDRESSED

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THAT BEFORE, AND I THINK IT'S IMPORTANT FOR THOSE OF US THAT ARE VOTING ON THIS TO PUT THESE PROJECTS INTO THAT PERSPECTIVE AS WELL. AGAIN, THANK YOU VERY MUCH, DR. SAMBRANO AND STAFF, FOR THIS PRESENTATION.

CHAIRMAN THOMAS: THANK YOU, MR. JUELSGAARD.

ANY OTHER PRELIMINARY COMMENTS BY MEMBERS OF THE BOARD BEFORE WE PROCEED TO PUBLIC COMMENT? HEARING NONE, PLEASE PROCEED. WE HAVE OUR FIRST GUEST HERE.

DR. LIEU: I'M DEBORAH LIEU. I'M FROM UNIVERSITY OF CALIFORNIA DAVIS. I AM THE PRINCIPAL INVESTIGATOR OF APPLICATION 9460. WE'RE FOCUSING OUR RESEARCH ON DEVELOPING PACEMAKER CARDIOMYOCYTES FROM HUMAN INDUCED PLURIPOTENT STEM CELLS UTILIZING THE MICROENVIRONMENT TO DRIVE THE DIFFERENTIATION TOWARD THIS PACEMAKING TYPE.

I WOULD LIKE TO TAKE THIS OPPORTUNITY TO ADDRESS SOME OF YOUR COMMENTS. MAJORITY OF THE COMMENTS, THE CONCERNS THAT REVIEWERS HAVE, REVOLVE AROUND THE NEED FOR THIS BIOPACEMAKER BECAUSE THE ELECTRONIC PACEMAKER SEEMS TO BE SUFFICIENT FOR THE PATIENTS. AND THERE HAVE BEEN RECENT ADVANCEMENTS IN REMOTE SENSING, WIRELESS PACEMAKER, BUT IT

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DOESN'T MATTER HOW SOPHISTICATED THESE MEDICAL DEVICES GET. THERE ARE CONCERN ISSUES ASSOCIATED WITH ELECTRONICS THAT CANNOT BE FIXED OR BE REDESIGNED TO CIRCUMVENT THESE PROBLEMS, SUCH AS THE REQUIREMENT FOR BATTERIES.

SO THESE DEVICES WILL REQUIRE BATTERY REPLACEMENT EVERY FIVE TO EIGHT YEARS AT $40,000 PER SURGERY. AND THESE DEVICES ARE ALSO SUBJECT TO MAGNETIC INTERFERENCES. SO THESE ARE PROPERTIES ASSOCIATED WITH ELECTRONIC DEVICES THAT CANNOT -- THAT'S NOT REALLY FIXABLE. SO THERE'S REALLY A NEED FOR THIS BIOPACEMAKER TO GET AROUND THESE ISSUES.

AND, IN ADDITION, BABIES AND CHILDREN WITH FAST GROWTH IN HEART SIZE AND WHO HAVE SMALLER BLOOD VESSELS ARE REALLY NOT IDEAL CANDIDATES TO RECEIVE THESE ELECTRONIC PACEMAKERS.

AND, LASTLY, I JUST WANT TO POINT OUT THIS AREA WITH CARDIAC ARRYTHMIA HAS NOT REALLY BEEN THAT WELL FUNDED COMPARED TO SOME OF THE OTHER CARDIAC ISSUES. THANK YOU FOR CONSIDERING MY APPLICATION.

CHAIRMAN THOMAS: THANK YOU VERY MUCH. NEXT PLEASE.

DR. SNEDDON: SO HELLO. MY NAME IS JULIE SNEDDON, AND I'M AT UCSF, AND I'M THE PI ON 09635. AND I JUST WANTED TO BRIEFLY THANK EVERYONE FOR THE

OPPORTUNITY TO SPEAK AND ALSO THANK THE GWG FOR THEIR FAVORABLE COMMENTS ON OUR PROPOSAL, AS WELL AS THEIR UNANIMOUS RECOMMENDATION FOR FUNDING.

I DID WANT TO JUST POINT OUT WHAT I THINK ARE THE KEY BOTTLENECKS THAT OUR PROPOSAL ADDRESSES IN THIS FIELD. THE GOAL OF OUR PROPOSAL, AS YOU JUST HEARD, IS TO DESIGN A CELLULAR THERAPEUTIC TO CURE TYPE 1 DIABETES. AND AS MANY PEOPLE IN THIS ROOM KNOW, THERE HAVE BEEN A NUMBER OF DIFFERENT PROTOCOLS OR STRATEGIES THAT PEOPLE HAVE RECENTLY DEVISED TO GENERATE A PANCREATIC BETA CELL FROM A STEM CELL, BUT THERE'S A NUMBER OF IMPORTANT CHALLENGES THAT STILL REMAIN. AND I'LL HIGHLIGHT JUST A FEW OF THEM THAT I THINK THAT OUR PROPOSAL REALLY ADDRESSES.

SO THE FIRST IS THE FUNCTION OF THESE CELLS. WE'RE NOT QUITE THERE IN TERMS OF REALLY HAVING A BONA FIDE BETA CELL. THE SECOND IS THE SURVIVAL AND ENGRAFTMENT OF THOSE CELLS ONCE THEY GET INTO A PATIENT, WHICH IS OBVIOUSLY VERY IMPORTANT. THE THIRD RELATES TO THE BATCH-TO-BATCH VARIATION THAT PLAGUES, I THINK, MANY OR ALL OF US WHO ARE DOING THESE TYPES OF DIFFERENTIATION EXPERIMENTS. AND THE FOURTH IS THE STABILITY OF THAT CELL. ONCE YOU MAKE THAT STEM CELL, DOES IT

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STAY THE PANCREATIC BETA CELL? DOES IT STAY WHAT IT'S SUPPOSED TO BE, OR DOES IT REVERT BACK TO SOME OTHER TYPE OF LESS DIFFERENTIATED PRODUCT?

SO WHAT WE'VE DONE IS WE'VE GONE BACK TO THE BIOLOGY ESSENTIALLY AND SAID, HOW DO THESE BETA CELLS GROW UP? HOW DO THEY FORM IN THE HUMAN BEING DURING DEVELOPMENT? AND IT TURNS OUT THAT, LIKE ANY CELL IN YOUR BODY, THEY DON'T EXIST IN ISOLATION. THEY REALLY CO-DEVELOP IN THE MICROENVIRONMENT OR A CELLULAR NICHE.

SO THAT'S THE FOCUS OF OUR PROPOSAL. WE'VE DEVISED A NOVEL METHOD FOR CREATING THE WHOLE SORT OF MICROENVIRONMENT AROUND THAT BETA CELL. AND WE BELIEVE, GIVEN THE STRENGTH OF OUR PRELIMINARY DATA, THAT THAT'S A VERY SUCCESSFUL STRATEGY THAT HAS ALREADY ALLOWED US TO OVERCOME A NUMBER OF THOSE KEY CHALLENGES I JUST MENTIONED TO YOU. SO WE HAVE A MORE STABLE CELL. THESE CELLS PERSIST IN VITRO OR IN A DISH FOR MANY WEEKS AT LEAST. WE HAVE IMPROVED FUNCTION AND IMPROVED SURVIVAL ONCE THEY GET INTO THE PATIENT. AND WE'VE ALSO SEEN MUCH MORE UNIFORMITY IN THE PRODUCTION OF THESE CELLS AS A RESULT. SO I THINK IT KIND OF MAKES SENSES THAT ONCE YOU MORE CLOSELY RECAPITULATE IN THE BODY AND USE TISSUE ENGINEERING STRATEGIES TO RECAPITULATE

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THAT IN A DISH, THAT YOU'RE GOING TO GET SOMETHING THAT IS MUCH CLOSER TO WHAT WE WANT.

IN SUMMARY, I THINK WE BELIEVE THAT RATHER THAN JUST PUTTING IN BETA CELLS ALONE OR PROGENITORS ALONE, AS HAVE BEEN TRIED, WE BELIEVE THAT THE OPTIMAL THERAPEUTIC BENEFIT FOR TYPE 1 DIABETES WILL REALLY COME WHEN WE HAVE THESE TISSUE ENGINEERING STRATEGIES LIKE WE PROPOSE. I JUST WANT TO THANK YOU AGAIN FOR THE OPPORTUNITY TO SPEAK.

CHAIRMAN THOMAS: THANK YOU. NEXT PLEASE.

DR. DESAI: WE'RE SHARING TIME. MY NAME IS TEJEL DESAI. I'M FROM UCSF. AND THIS IS MATTHIAS HEBROK, AND WE'RE REPRESENTING THE PROPOSAL THAT IS FOCUSED ON A THIN FILM ENCAPSULATION DEVICE FOR DELIVERY OF STEM CELL-DERIVED INSULIN-PRODUCING CELLS.

ONE OF THE THINGS THAT I WANTED TO TALK ABOUT IS THE IMPORTANCE OF ONCE WE HAVE STEM CELLS, HOW ARE WE GOING TO DELIVER THEM? AND WE'RE REALLY FOCUSING ON THINKING ABOUT A NEW STRATEGY FOR MACROENCAPSULATION. WE REALIZE THAT CIRM HAS LOOKED AT THESE TECHNOLOGIES BEFORE AND SUPPORTED THEM. IN FACT, THERE HAVE BEEN SOME INVESTMENTS EVEN AT THE CLINICAL STAGE, BUT THERE ARE SOME REAL BOTTLENECKS IN TERMS OF REALLY INTRODUCING A DEVICE THAT NOT

ONLY PROTECTS THE CELLS AND SORT OF HOUSES THEM, BUT REALLY ENHANCES THEIR FUNCTION THAT ALLOWS THEM TO ENGRAFT AND MAINTAIN LONG-TERM VIABILITY. I THINK WE'VE SEEN THROUGHOUT THE COURSE OF MANY STUDIES THAT HAVE BEEN CONDUCTED THAT IF WE DON'T ADDRESS THOSE, THERE WILL BE A REAL CHALLENGE TO TRANSLATION AND HOW WE GET CELLS TO THE PATIENT. SO OUR PROPOSAL FOCUSES ON A BIOENGINEERED DEVICE THAT NOT ONLY SECRETES FACTORS THAT ENHANCE CELL VIABILITY LONG TERM, BUT ALSO IMMUNOMODULATE THE LOCAL ENVIRONMENT SUCH THAT WE CAN REALLY PROVIDE A PROTECTIVE BARRIER. I HAND OVER TO MY COLLEAGUE.

DR. HEBROK: I'M MATTHIAS HEBROK FROM UCSF. I'M ACTUALLY DIRECTOR OF THE DIABETES AT THE UCSF'S, SO WE'VE BEEN DOING THIS FOR A LONG TIME.

MY PART OF THIS PROJECT, WHICH I THINK IS THE CONSOLIDATION OF THREE IMPORTANT PIECES. ONE IS BIOENGINEERING, WHICH TEJAL IS TALKING ABOUT. AND THE OTHER ONE IS ABOUT IMMUNOLOGY, WHICH QIZHI TANG IS DOING, AND WE ARE PRODUCING THE CELLS FROM STEM CELLS. THIS IS A VERY FAST-MOVING FIELD. DR. SAMBRANO HAS POINTED OUT THAT CIRM HAS ALREADY INVESTED IN THIS KIND OF TECHNOLOGY. LET ME JUST SAY THAT OVER THE LAST SIX MONTHS, MY LAB HAS NOW GENERATED BETA CELLS FROM STEM CELLS. THEY'RE 92

PERCENT IDENTICAL TO THE ONES THAT ALL OF YOU GUYS HAVE IN YOUR BODY. THIS IS SOMETHING THAT NO ONE HAS ACHIEVED AS OF YET, AT LEAST NOT PUBLISHED IN THE PUBLICATIONS THAT WE CAN GET. AND WE THINK THAT THESE CELLS ARE READY TO GO. WE ACTUALLY HAVE DATA AND WE HAVE SUBMITTED MORE DATA IN RESPONSE TO VERY GOOD REVIEWERS THAT THEY'VE GOTTEN AND TO SHOW THAT THESE CELLS ARE READY TO SECRETE INSULIN IN THE WAY THAT NORMAL HUMAN BETA CELLS DO IT, AND THEY LAST.

DR. DESAI: THANK YOU VERY MUCH. HOPEFULLY OUR COMMENTS ALSO ADDRESS THIS.

CHAIRMAN THOMAS: THANK YOU. NEXT PLEASE.

DR. PORTEUS: HI. THANK YOU. MY NAME IS MATT PORTEOUS. I'M THE PI ON 9637, THE PROJECT ON GENOME EDITING FOR CYSTIC FIBROSIS. I WANT TO THANK YOU ALL FOR ALLOWING US TO PRESENT. THERE'S GOING TO BE THREE OF US. I'LL TALK BRIEFLY AND THEN INTRODUCE MY TWO SPEAKERS.

SO BRIEFLY TO SUPPLEMENT WHAT WAS SUBMITTED AS A WRITTEN SUPPLEMENT AND TO ADDRESS THE CONCERNS IS THAT WE NOW HAVE DATA THAT WE CAN ACTUALLY EFFICIENTLY MODIFY THE CFTR GENE IN AIRWAY STEM CELLS FROM CYSTIC FIBROSIS PATIENTS. AND THAT PROGRESS HAS BEEN MADE SINCE THE SUBMISSION OF OUR PROPOSAL.

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IN ADDITION, WITH OUR COLLABORATORS DR. POE AND AMIN, HERE WE HAVE NOW SHOWN THAT USING A THREE DIMENSIONAL SCAFFOLD WE CAN EXPAND THESE CELLS BY A THOUSANDFOLD. SO NOW WE HAVE THE ABILITY TO BOTH CHARACTERIZE AND EXPAND THE CELLS PRIOR TO IMPLANTATION.

WE RECOGNIZE THAT ENGRAFTMENT OF THESE CELLS IS GOING TO BE ONE OF THE KEY FEATURES TO OUR PROGRAM. WE RECOGNIZE THE CHALLENGES AND HAVE ALREADY INITIATED STUDIES AND HAVE PROPOSED SOME BACKUP STRATEGIES, INCLUDING THE POSSIBILITY OF USING SCAFFOLDS TO IMPLANT THESE CELLS IN THE AREA WE WANT.

THE FINAL CONCERN THAT I'LL ADDRESS IS THE ONE ABOUT WHY ARE WE DOING THE SINUS VERSUS THE LUNG. WE RECOGNIZE THAT CYSTIC FIBROSIS IS PRIMARILY A LUNG DISEASE, BUT MY TWO COLLEAGUES ARE GOING TO ADDRESS THE IMPORTANCE OF SINUS DISEASE, BUT THE OTHER REASON WE'RE CHOOSING THE SINUSES IS IT'S AN ACCESSIBLE AND SAFE SITE TO ESTABLISH PROOF OF CONCEPT ON HOW YOU WOULD MODIFY A CELL AND GET IT ENGRAFTED IN THE AIRWAY EPITHELIUM WITHOUT HAVING TO DEAL WITH BOTH THE RISK AND CHALLENGES OF GETTING DEEP INTO THE LUNG IN CYSTIC FIBROSIS PATIENTS.

SO WITH THAT, I'M GOING TO INTRODUCE DR.

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NAYAK, WHO IS ONE OF OUR TEAM, WHO'S AN OTORHINOLARYNGOLOGIST AND TREATS PATIENTS WITH CYSTIC FIBROSIS SINUS DISEASE.

DR. NAYAK: PLEASURE TO MEET EVERYONE. SO I'M ONE OF THE SURGEON SCIENTISTS AT STANFORD, AND I EXCLUSIVELY DO SINUS SURGERY FOR A LIVING. I'M HAPPY TO ANSWER ANY QUESTIONS ABOUT ENGRAFTMENT, ACQUISITION OF CELLS, REIMPLANTATION OF THE CELLS IF THERE'S INTEREST AND TIME LATER.

BUT CYSTIC FIBROSIS IS A SINISTER, AND IT'S A DEADLY DISEASE OF CHILDHOOD AND OF YOUNG ADULTS. AND YOU WOULDN'T REALLY KNOW THAT IF YOU DIDN'T MEET SOMEONE LIKE CAMERON, WHO'S A PATIENT OF A MINE WHO'S BEEN IN MY PRACTICE FOR ABOUT FIVE, SIX YEARS NOW. BUT HE'S A SENIOR IN COLLEGE, BUT HE'S ALREADY HAD SEVEN SINUS SURGERIES IN NINE YEARS.

IT'S A DISEASE THAT LEAVES SCARRING, RECURRENT INFECTIONS, AND NUMEROUS ISSUES WITH THE UPPER AIRWAY, THE SINUSES, THAT DOES AFFECT THE LOWER AIRWAY, AS HE'LL TELL YOU. BUT I APPRECIATE YOU TAKING TIME OUT OF YOUR SENIOR YEAR OF COLLEGE TO COME AND JOIN US.

CAMERON: AS DR. NAYAK SAID, MY NAME IS CAMERON (INAUDIBLE), 22 YEARS OLD AND CYSTIC FIBROSIS. CYSTIC FIBROSIS IS A GENETIC DISEASE THAT

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AFFECTS HUNDREDS OF THOUSANDS OF PEOPLE AROUND THE WORLD WITH A LIFE EXPECTANCY OF ABOUT 37 YEARS. IT AFFECTS THE RESPIRATORY AND DIGESTIVE SYSTEMS OF THE BODY, BUT IT ALSO IMPACTS THE LIVER AND SINUSES, WHICH IS THE MAIN REASON WHY THIS RESEARCH IS SO IMPORTANT.

I'VE BEEN TO COUNTLESS DOCTORS' VISITS OVER JUST ONE YEAR. I HAVE AT LEAST ONE HOSPITALIZATION EVERY YEAR FOR VARIOUS LUNG EXACERBATIONS WHEN THEY OCCUR. BUT AS IT'S BEEN DISCUSSED, THE LUNGS ARE THE MAIN ISSUE THAT IS USUALLY BROUGHT UP WITH CYSTIC FIBROSIS. HOWEVER, THE CAUSE MOST OF MY LUNG ISSUES IS ACTUALLY THE SINUSES. AS DR. NAYAK BRIEFLY POINTED OUT, IF THERE'S A SINUS INFECTION, THAT CAN EASILY GO DOWN AND SPREAD INTO THE AIR PASSAGEWAYS, AND THAT CAUSES TISSUE DAMAGE AND DAMAGING OF THE LUNGS IN GENERAL.

SO THIS RESEARCH THAT THESE DOCTORS ARE DOING IS JUST AMAZING AND SOUNDS LIKE IT CAN REALLY PROLONG MY LIFE AND ESPECIALLY MY QUALITY OF LIFE BECAUSE HAVING SINUS INFECTIONS CONSTANTLY IS NO FUN. ALWAYS IN PAIN AND THERE'S NOT REALLY ANYTHING YOU CAN DO. THERE HASN'T REALLY BEEN ANY BREAKTHROUGH IN RECENT HISTORY FOR CF IN GENERAL, BUT ESPECIALLY THE SINUSES. AND AS WELL AS THIS,

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THIS IS RESEARCH WHERE THEY REMOVE STEM CELLS FROM THE NOSE AND THEN CORRECT THEM, IMPLANT THEM BACK INTO THE SINUS CAVITIES, IT CAN RESTORE SINUSES TO NORMAL FUNCTION, WHICH WOULD BE AMAZING.

SO ON BEHALF OF THE WHOLE CF COMMUNITY, I JUST WANT TO SAY HOW MUCH THIS RESEARCH REALLY WOULD HELP EVERY CF PATIENT OUT THERE. THANK YOU FOR GIVING ME THE OPPORTUNITY TO SPEAK.

MR. TORRES: DOCTOR, I WANTED TO ASK YOU A QUESTION. I KNOW HOW SERIOUS THIS DISEASE IS. DURING MY COLON CANCER RECOVERY, A NEXT DOOR NEIGHBOR OF MINE WAS A YOUNG WOMAN WHO HAD BEEN IN THERE EIGHT TIMES. MY HEART JUST WENT OUT TO HER AND OBVIOUSLY THE WHOLE CF COMMUNITY.

WHAT PERCENTAGE OF THE PATIENTS STATEWIDE DO YOU ASSUME FIT INTO THE SINO CATEGORY AS OPPOSED TO THE LUNG CATEGORY?

DR. NAYAK: 100 PERCENT OF PATIENTS WHO HAVE CYSTIC FIBROSIS HAVE SOME LEVEL OF SINUSITIS.

MR. TORRES: WE THOUGHT IT WAS ALL JUST THE LUNG.

DR. NAYAK: RIGHT. SO IT'S A SPECTRUM LIKE ANYTHING ELSE. SOME PEOPLE HAVE SEVERE DIABETES AND SOME PEOPLE HAVE MILD DIABETES. CYSTIC FIBROSIS, I HAVE SOME PATIENTS WHO HAVE NEVER HAD

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SINUS SURGERY, BUT THEY NEED SINUS TREATMENT, SINUS RINSES, ANTIBIOTICS OCCASIONALLY FOR THE SINUSES, AND MANY, UNFORTUNATELY LIKE CAMERON, HE HAD HIS FIRST SINUS SURGERY AT 12 YEARS OLD, AND HE'S HAD RECURRENT POLYPS, RECURRENT SCARRING, INFECTIONS, MORE AND MORE ADVANCES SURGERIES. NOW HE'S ON NO. 7, AND THE LAST ONE WAS A FEW MONTHS AGO.

MR. TORRES: WHAT'S YOUR MAJOR?

CAMERON: CHEMICAL ENGINEERING.

CHAIRMAN THOMAS: NEXT PLEASE.

DR. BACCHETTA: HI. MY NAME IS ROSA BACCHETTA, AND I AM FROM STANFORD, AND I AM THE PI OF THE 9526, GENE EDITING FOR FOXP3 IN HUMAN STEM CELLS. AND THANK YOU VERY MUCH FOR THE INTERACTION AND FOR THE RECOGNITION OF THIS.

I JUST LIKE TO POINT OUT A COUPLE OF THINGS. ONE IS THAT WE DEVOTED MANY YEARS IN THE PAST CLINICAL AND RESEARCH STUDIES IN IPEX SYNDROME, WHICH IS A SERIES OF DISEASES WITH AUTOIMMUNITY OF GENETIC ORIGIN AFFECTING CHILDREN VERY, VERY EARLY IN LIFE. SO THIS, I BELIEVE, IS A UNIQUE EXPERTISE TO DEVELOP A DEFINITIVE CURE FOR THE DISEASE AND ALSO GIVE US ACCESS INTERNATIONALLY TO RECRUIT PATIENT AND TO RECRUIT PATIENT CELLS FOR THE STUDIES, WHICH, THEREFORE, THIS IS MINIMIZING THE

LIMITATION OF TARGETING AREA OF DISEASE.

AND ON TOP OF THIS FOXP3 IS THE CAUSE OF THIS GENE. ITS EXPRESSION IS VERY UNIQUE, HIGHLY REGULATED, AND ALSO VERY DIVERSELY REGULATED IN DIFFERENT CELL TYPES (UNINTELLIGIBLE) AND, THEREFORE, THE GENOME EDITING APPROACH THAT WE PROPOSE IS REALLY A UNIQUE TECHNOLOGY THAT COULD PROVIDE CURE OF THE DISEASE AND RESTORATION OF THE FUNCTIONS OF THE STEM CELLS IN THESE PATIENTS. THEREFORE, WE HAVE ALL THE SAFETY AND EFFICACY TO TEST THESE CELLS IN THE LAB. AND, THEREFORE, I BELIEVE IN THE NEXT TWO YEARS WITH THIS WORK WE PROVIDE THE OPPORTUNITY FOR THE CURE OF THIS DISEASE AND ALSO OF THE OTHER DISEASES OF THE IMMUNE SYSTEM GENETICALLY. THANK YOU.

CHAIRMAN THOMAS: THANK YOU VERY MUCH. ANY OTHER MEMBERS OF THE PUBLIC WHO WOULD LIKE TO COMMENT AND HAVEN'T THUS FAR? ANY OTHER MEMBERS OF THE PUBLIC AT ANY OF THE OTHER SITES ON THE PHONE THAT WOULD LIKE TO COMMENT AT THIS POINT? HEARING NONE, WE'LL NOW PROCEED TO CONSIDERATION OF THESE PROPOSALS IN PROGRAMMATIC REVIEW. AND I WILL AT THIS POINT TURN THE MEETING OVER TO DR. PRIETO.

DR. PRIETO: THANK YOU, J.T. CAN EVERYONE HEAR ME CLEARLY?

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SO FIRST OF ALL, I JUST WOULD LIKE TO REMIND EVERYONE THAT ANY MEMBERS WHO HAVE AN INTEREST IN AN APPLICATION IN TIER I OR TIER II WILL NOT BE ABLE TO PARTICIPATE IN DISCUSSING AND VOTING ON A MOTION THAT AFFECTS THOSE APPLICATIONS. SO THAT SAID, I WOULD FIRST LIKE TO REMIND EVERYONE WE ARE A LITTLE SHORT ON TIME. WE ONLY HAVE ABOUT 45 MINUTES LEFT, AND WE HAVE RUN OUT OF TIME BEFORE. SO IN THE INTEREST OF EFFICIENCY, I'D LIKE TO MOVE THROUGH THIS AS QUICKLY AS WE CAN AND FIRST CONSIDER A MOTION TO MOVE ANY APPLICATIONS FROM TIER II UP TO TIER I. ARE THERE ANY MOTIONS?

HEARING NONE, I'D LIKE TO CONSIDER A MOTION NOT TO FUND THE APPLICATIONS THAT REMAIN IN TIER II.

MR. TORRES: SO MOVED.

MS. WINOKUR: SECOND.

DR. PRIETO: OKAY. MOVED AND SECONDED. I'M NOT SURE WHO THAT WAS, BUT WERE THOSE BOTH AT CIRM?

MS. BONNEVILLE: IT WAS ART AND DIANE.

DR. PRIETO: OKAY. THANK YOU. OKAY. CAN WE HEAR A VOTE? WILL WE NEED TO CALL THE ROLL? I PRESUME.

MR. TOCHER: HI, FRANCISCO. THIS IS SCOTT

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TOCHER. YES. WE'LL MAKE A ROLL CALL VOTE. AND FOR THOSE WHO MAY HAVE A CONFLICT WITH ANY APPLICATION WITHIN TIER II, PLEASE INDICATE YOUR VOTE AYE OR NAY EXCEPT WITH RESPECT TO THOSE APPLICATIONS THAT YOU HAVE A CONFLICT.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: AYE.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: SHERRY LANSING. KATHY LAPORTE. LAUREN MILLER.

MS. MILLER: AYE.

MS. BONNEVILLE: ADRIANA PADILLA. JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: AYE.

MS. BONNEVILLE: JEFF SHEEHY. OS STEWARD.

DR. STEWARD: YES.

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MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
DR. PRIETO: I WOULD NEXT LIKE TO CONSIDER A MOTION TO FUND THOSE APPLICATIONS IN TIER I THAT RECEIVED A UNANIMOUS RECOMMENDATION FOR FUNDING FROM THE GWG.
MR. TORRES: SO MOVED.
DR. PRIETO: EVERYBODY GIVING A RANK SCORE GAVE THIS A SCORE OF 85 OR ABOVE.
MR. ROWLETT: I'LL SECOND ART'S MOTION.
DR. PRIETO: OKAY. I DIDN'T HEAR ART'S MOTION, BUT THANK YOU VERY MUCH, SENATOR TORRES.
MR. TORRES: I'LL REPEAT IT.
DR. PRIETO: OKAY. ALL RIGHT. ANY DISCUSSION?
DR. JUELSGAARD: SO REMIND ME WHAT THE MOTION AGAIN IS.
DR. PRIETO: OKAY. THE MOTION IS TO FUND JUST THOSE APPLICATIONS IN TIER I THAT RECEIVED A UNANIMOUS RECOMMENDATION FOR FUNDING FROM THE GWG.

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DR. JUELSGAARD: SO THAT WOULD INCLUDE, AMONGST OTHERS, 09596 AND 09615; IS THAT RIGHT?

MS. BONNEVILLE: YES.

CHAIRMAN THOMAS: STEVE, IT LOOKS LIKE IT WOULD INCLUDE SEVEN OF THE 14 PROJECTS.

DR. JUELSGAARD: I UNDERSTAND, BUT I'M FOCUSING ON TWO SPECIFICALLY.

MS. BONNEVILLE: YES.

DR. JUELSGAARD: ALL RIGHT. THEN I MOVE TO AMEND SENATOR TORRES' MOTION TO APPROVE ALL BY REMOVING FROM THAT GROUP APPLICATIONS 09596 AND 09615 ON THE BASIS THAT WE HAVE ALREADY INVESTED, IN THE FIRST CASE, $61.6 MILLION IN ONGOING PROJECTS IN THE SAME AREA, AND THE SECOND $83.1 MILLION IN PROJECTS IN THE SAME AREA.

MS. WINOKUR: SECOND.

MR. TOCHER: WILL THE MAKER OF THE MOTION ACCEPT AN AMENDMENT?

MR. TORRES: NO. I WON'T ACCEPT IT. I THINK WE NEED TO DO THIS RESEARCH. YOU CAN DO A SUBSTITUTE MOTION AFTER THE VOTE.

DR. PRIETO: SCOTT, HOW DO WE PROCEED WITH THIS IF WE HAVE TWO COMPETING MOTIONS?

MR. TOCHER: FIRST OF ALL, SENATOR TORRES' MOTION HAS THE FLOOR. IF DR. JUELSGAARD WOULD LIKE

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TO MAKE AN AMENDMENT, WHICH I THINK HE'S OFFERED AND SENATOR TORRES HAS DECLINED, THEN WE'LL PROCEED WITH A VOTE AND A DISCUSSION ON SENATOR TORRES' MOTION WHICH HAS BEEN SECONDED.

DR. PRIETO: OKAY. I'LL ASK FOR DISCUSSION ON THE ORIGINAL MOTION.

DR. JUELSGAARD: I JUST WILL REPEAT WHAT I SAID. THE TWO PARTICULAR APPLICATIONS, THE ONE ENDING IN 96 AND THE OTHER ENDING IN 15, HAVE ALREADY, THESE TWO PARTICULAR AREAS, AND THIS REVOLVES AROUND, THE FIRST, THE HEART, THE CARDIOVASCULAR AREA, AND THE SECOND AROUND TREATMENT OF CANCER, BOTH HAVE RECEIVED, BOTH AREAS, BOTH THERAPEUTIC AREAS HAVE RECEIVED SUBSTANTIAL FUNDING FROM THIS ORGANIZATION FOR A NUMBER OF PROJECTS. IF WE APPROVE SOMETHING THAT WE'VE ALREADY INVESTED A HUGE AMOUNT OF MONEY IN WILL ULTIMATELY MEAN THAT THERE MAY BE PROJECTS WHICH HAVE RECEIVED VERY LITTLE FUNDING WHICH FALL FARTHER DOWN ON THE LIST, ALTHOUGH THEY ARE ABOVE -- THEY'RE IN THE TIER I GROUP AND, THEREFORE, ELIGIBLE FOR OUR FUNDING, THERE WILL BE PROJECTS THAT WON'T GET FUNDED THAT HAVEN'T HAD THE KIND OF SUPPORT THAT THESE TWO AREAS HAVE.

I THINK ONE OF THE OBLIGATIONS, ONE OF THE

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REASONS WE'RE EVEN DOING WHAT WE'RE DOING IS NOT SIMPLY TO SAY, GOOD JOB, GWG, ON THE SCIENCE SIDE OF THESE PROJECTS. OURS IS TO LOOK MORE DEEPLY AT AN ISSUE THAT NORMALLY THE GWG DOESN'T LOOK AT, WHICH IS PROGRAMMATIC REVIEW. THEY DON'T SEE THESE NUMBER OF ACTIVE PROJECTS AND THE AMOUNTS OF MONEY THAT WE'VE INVESTED LIKE WE DO. THAT'S WHY THIS INFORMATION IS HERE.

SO WE HAVE A DECISION, A SECOND DECISION, TO MAKE WHICH IS BEYOND THE SCIENTIFIC MERIT, AND THAT IS HOW DO WE REALLY WANT TO SPEND OUR MONEY, PARTICULARLY IN THESE YEARS WHEN WE HAVE WANING RESOURCES. AND WHEN WE HAVE AREAS THAT HAVE ALREADY RECEIVED OVER TIME SUCH SUBSTANTIAL SUPPORT VERSUS AREAS WHICH HAVE RECEIVED VERY LITTLE SUPPORT, IT SEEMS TO ME INCUMBENT UPON US TO REALLY TAKE THAT INTO ACCOUNT. THAT'S WHY I AMENDED, WHICH WAS REJECTED, THE MOTION TO JUST APPROVE ALL OF THESE IN ONE BIG BUCKET, WHICH I THINK IS NOT QUITE THE RIGHT WAY TO DO IT, BUT IN ANY EVENT TWO OF THEM THAT I THINK HAVE HAD MORE THAN THEIR FAIR SHARE OF ATTENTION IN HOPES THAT WE'LL APPROVE OTHERS THAT HAVEN'T HAD THAT KIND OF ATTENTION.

DR. PRIETO: ANY OTHER COMMENTS?

DR. STEWARD: I DON'T THINK I'M IN

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CONFLICT ON THIS MOTION; IS THAT CORRECT?

MR. TOCHER: INCORRECT, OS. RIGHT NOW YOU HAVE APPLICATIONS THAT ARE IN TIER I RIGHT NOW.

DR. STEWARD: THIS DOESN'T INVOLVE TIER I. THIS INVOLVES ONLY THE ONES THAT WERE UNANIMOUSLY RECOMMENDED FOR FUNDING.

MR. TOCHER: YOU ARE CORRECT; HOWEVER, THOSE APPLICATIONS ARE IN TIER I. AND YOU HAVE AN APPLICATION THAT IS IN TIER I. AND AS A RESULT, YOU CANNOT PARTICIPATE IN THE DISCUSSION AND VOTE ON THESE.

DR. STEWARD: GOT IT. THANK YOU.

DR. PRIETO: SCOTT, I'D LIKE TO MAKE A COMMENT JUST AS SOMEONE WHO PARTICIPATED IN THE REVIEW AND ALSO OBVIOUSLY HERE. BUT PART OF THE PROGRAMMATIC ISSUE, I THINK, IS THE DISEASE IMPACT. AND I THINK THAT IT'S WORTH CONSIDERING THAT WE'RE TALKING ABOUT APPLICATIONS THAT AFFECT REFRACTORY CANCER AND CARDIOVASCULAR DISEASE. I THINK IT IS A PROGRAMMATIC ISSUE THAT THESE DISEASES ARE CONDITIONS THAT AFFECT MILLIONS AND MILLIONS OF PEOPLE. SO THAT IT'S PERHAPS APPROPRIATE FROM A PROGRAMMATIC POINT OF VIEW THAT WE INVEST A LOT OF OUR RESOURCES IN DIFFERENT METHODS TO FIND A SOLUTION TO THOSE PROBLEMS.

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DR. HIGGINS: COULD I MAKE A COMMENT?

DR. PRIETO: PLEASE.

DR. HIGGINS: I FULLY APPRECIATE WHAT STEVE IS TRYING TO DO, AND I APPLAUD HIS EFFORTS. I DON'T CRITICIZE THEM WHATSOEVER, BUT I GUESS I WOULD DRAW THE DISTINCTION BETWEEN SATURATED FUNDING FOR AN AREA VERSUS SUBSTANTIAL FUNDING FOR AN AREA. I THINK I WOULD TAG ONTO FRANCISCO'S COMMENTS THAT CANCER AND HEART DISEASE ARE SUCH HUGE INDICATIONS, AND I DON'T THINK THAT WE, CIRM, HAVE SATURATED THE POSSIBILITIES OF CURES AND TREATMENTS THAT WE CAN SAY THAT WE'VE GIVEN ENOUGH MONEY TO ANY OF THOSE. OBVIOUSLY WE WISH WE COULD GIVE MONEY TO EVERYBODY, BUT OBVIOUSLY THAT'S NOT THE POSSIBILITY.

SO I GUESS I WOULD ARGUE THAT THIS IS NOT A SATURATED AREA OF OUR FUNDING; AND, THEREFORE, I WOULDN'T EXCLUDE IT FROM ADDITIONAL FUNDING. THAT'S MY COMMENT.

DR. PRIETO: OKAY. IF THERE ARE NO MORE COMMENTS, I THINK IT'S ALMOST 11:30 AND WE HAVE SEVERAL OTHER APPLICATIONS WE'RE GOING TO APPROACH INDIVIDUALLY. SO I'D LIKE TO CALL FOR A VOTE.

DR. JUELSGAARD: FRANCISCO, COULD I MAKE ONE MORE QUICK COMMENT BEFORE WE DO THAT?

DR. PRIETO: GO AHEAD, STEVE.

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MR. JUELSGAARD: SO I WOULD JUST POINT OUT THAT IF WE FOLLOW THIS APPROACH, THERE ARE PROJECTS THAT ARE AT THE BOTTOM END OF THE PROGRAM THAT DEAL WITH PARKINSON'S DISEASE, TRAUMATIC BRAIN INJURY, CYSTIC FIBROSIS, THINGS LIKE THAT. I'M NOT SAYING IT'S PREDETERMINED THAT WE WON'T FUND THOSE, BUT AT SOME POINT WE'RE NOT GOING TO HAVE THE MONEY TO FUND OTHER PROGRAMS. SO BY AGREEING TO DO WHAT WE'RE DOING, WE'RE BASICALLY ALSO AGREEING THAT WE'RE GOING TO DROP SOME PROGRAMS ALONG THE WAY THAT AREN'T GOING TO GET FUNDED THAT HAVE PRIME NEEDS AS WELL.

DR. PRIETO: I THINK THAT'S ALWAYS GOING TO BE TRUE.

MR. TOCHER: FRANCISCO, MAY I JUST RESTATE THE MOTION THEN?

DR. PRIETO: YES, PLEASE.

MR. TOCHER: SO THIS IS A MOTION TO FUND THOSE APPLICATIONS WHICH HAVE RECEIVED A UNANIMOUS TIER I SCORE FROM THE GRANTS WORKING GROUP. THOSE CONSIST OF THE TOP FOUR APPLICATIONS, WHICH ARE 9526, 9649, 9565, AND 9615, AND THREE MORE APPLICATIONS, WHICH ARE 9624, 9596, AND 9635.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

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MS. BONNEVILLE: DAVID HIGGINS.
DR. HIGGINS: YES.
MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: NO.
MS. BONNEVILLE: LAUREN MILLER.
MS. MILLER: YES.
MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: YES.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: AYE.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
DR. PRIETO: OKAY. THAT MOTION HAVING BEEN APPROVED, I WOULD LIKE TO CONSIDER THE REMAINING APPLICATIONS IN TIER I IN RANK ORDER UNTIL WE HAVE REACHED OUR BUDGET CAP. SO CAN I HEAR A

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MOTION -- IF I'M SEEING THE SLIDE CORRECTLY -- WELL, ACTUALLY WHY IS 9569 ALSO IN GREEN?

DR. SAMBRANO: SO WHAT I'M SHOWING IN THE SPREADSHEET IN BLUE NOW ARE THOSE THAT HAVE BEEN APPROVED BY EACH OF THE MOTIONS. SO THE ONES IN BLUE ARE NOW THE ONES THAT FROM THE PREVIOUS MOTION WERE UNANIMOUS AND, THEREFORE, NOW APPROVED FOR FUNDING. AND THE TOTAL OF THE AMOUNT THAT HAS BEEN APPROVED IS IN THE TOP LEFT-HAND CORNER. AND SO THE ONE THAT'S IN GREEN, 9569, WAS NOT UNANIMOUSLY RECOMMENDED. SO, THEREFORE, THAT'S THE NEXT ONE THAT, BASED ON THE DIRECTION YOU WANTED TO GO, WOULD BE THE ONE TO CONSIDER.

DR. PRIETO: YES. OKAY. THANK YOU FOR CLARIFYING THAT.

COULD I HEAR A -- SO 9569 IS THE HUNTINGTON'S DISEASE APPLICATION. CAN I HEAR A MOTION TO APPROVE?

DR. HIGGINS: SO MOVED.

DR. DULIEGE: I SECOND.

DR. PRIETO: ANY DISCUSSION? OKAY. HEARING NONE, CAN WE CALL THE ROLL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: AYE.

MS. BONNEVILLE: DAVID HIGGINS.

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DR. HIGGINS: YES.
MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: YES.
MS. BONNEVILLE: LAUREN MILLER.
MS. MILLER: YES.
MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: YES.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: AYE.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
MR. TORRES: SO, MR. CHAIRMAN, HOW MUCH DO WE HAVE LEFT?
DR. SAMBRANO: SO THE TOTAL THAT IS APPROVED THUS FAR IS 15.3 MILLION. AND YOU CAN SPEND UP TO 21.3.

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MS. BONNEVILLE: CAN WE PUT THE REMAINING. IF WE COULD JUST KEEP THAT RUNNING TOTAL OF WHAT'S LEFT, THAT WOULD BE GREAT.

MR. TORRES: SO WE HAVE 10 MILLION LEFT ON THE GREEN AREA, CORRECT?

MS. BONNEVILLE: NO. YOU HAVE ABOUT SIX MILLION LEFT ROUGHLY.

MR. TORRES: WE DON'T HAVE TO GO ON SERIATIM. WE COULD MAKE A MOTION FOR A PROJECT THAT MAY NOT BE NEXT.

MR. ROWLETT: GIVEN THE DIRECTION THAT FRANCISCO HAS RECOMMENDED, I WOULD NOT WANT TO DO THAT. I SUPPORTED FRANCISCO'S PROPOSAL TO GO IN RANK ORDER.

MR. TORRES: THAT'S FINE. I JUST WANTED TO POINT OUT WE COULD GO ANOTHER WAY AS WELL.

DR. PRIETO: THANK YOU. SO GOING IN RANK ORDER, THE NEXT APPLICATION, AND, OF COURSE, IF I DON'T HEAR A MOTION, THEN WE MOVE ON FOR ANY OF THESE, BUT I'D LIKE TO HEAR A MOTION ON APPLICATION NO. 09559.

CHAIRMAN THOMAS: FRANCISCO, THIS IS J.T. BEFORE YOU GET MOTIONS ON THAT, WE DO HAVE TO FACTOR IN THAT IF YOU GO THIS ROUTE, WHICH IS CERTAINLY A GOOD ROUTE, THAT THERE COULD BE PROJECTS FOR WHICH

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PROGRAMMATIC REVIEW DISCUSSION MIGHT PROCEED THAT YOU MAY NOT GET TO IF YOU MAXED OUT IN RANK ORDER.

DR. PRIETO: AGREED. ALTHOUGH WE ALSO, I THINK, SHOULD MENTION THAT THERE IS THE OPPORTUNITY FOR RESUBMISSION. IN FACT, AT LEAST TWO OF THE REMAINING APPLICATIONS HAVE ALREADY COME BACK AND BEEN RESUBMITTED AND GOTTEN RECOMMENDATIONS FOR FUNDING. BUT, YES, IF THERE IS DISCUSSION, FURTHER PROGRAMMATIC DISCUSSION, I THINK WE TALKED ABOUT SOME OF THAT WITH STEVE'S AMENDMENT.

MR. ROWLETT: I THINK THAT YOUR PROCESS ALSO ALLOWS FOR A PROPOSAL TO NOT BE APPROVED IN SPITE OF THE ORDER THAT IT MIGHT BE IN. SO JUST BECAUSE WE GO IN RANK ORDER DOES NOT MEAN THAT THE PROPOSAL WILL BE APPROVED BY US.

DR. PRIETO: THAT'S CORRECT.

I'M NOT HEARING ANY OTHER PROGRAMMATIC COMMENTS. CAN WE HEAR A MOTION ON 9559?

DR. HIGGINS: SO MOVED.

DR. PRIETO: AND A SECOND?

DR. JUELSGAARD: SECOND.

MS. MILLER: I'LL SECOND.

DR. PRIETO: OKAY. THANK YOU.

DISCUSSION?

I HAVE SOME COMMENT ALTHOUGH I'M NOT SURE

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THAT IT'S APPROPRIATE AS THE CHAIR. BUT, SCOTT.

MS. BONNEVILLE: YOU'RE FINE.

MR. TOCHER: GO AHEAD.

DR. PRIETO: THIS IS AN APPLICATION, ACTUALLY ONE OF THE SMALLER ONES IN THIS ROUND, FOR A DIFFERENT ENCAPSULATION DEVICE FOR THE TREATMENT OF TYPE 1 DIABETES. WE HAVE OBVIOUSLY INVESTED A CONSIDERABLE AMOUNT OF MONEY IN THIS AREA. IT IS AN AREA THAT IS FELT TO HAVE POTENTIALLY VERY HIGH IMPACT IN THE TREATMENT OF THIS DISEASE. ONE OF THE ISSUES IN THE PAST THAT WE'VE BEEN FOLLOWING PREVIOUSLY HAVE BEEN TECHNICAL ISSUES WITH THE ENCAPSULATION DEVICE. I THINK SOME OF THE SKEPTICISM -- THERE'S BEEN SOME SKEPTICISM IN THE GWG AS TO WHETHER ENCAPSULATION WILL WORK AT ALL, BUT CLEARLY PROOF OF CONCEPT HAS INDICATED THAT IT CAN, BUT THERE ARE PROBLEMS THAT NEED TO BE SOLVED. SO I THINK IT'S VALUABLE TO LOOK AT PEOPLE WHO ARE TRYING TO DO THIS IN A DIFFERENT WAY.

ANY OTHER COMMENTS OR QUESTIONS? CAN WE CALL THE ROLL?

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: I APPROVE.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: YES.
MS. BONNEVILLE: LAUREN MILLER.
MS. MILLER: YES.
MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: YES.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
MR. TORRES: MR. CHAIRMAN, MOVE TO APPROVE THE PARKINSON'S.
DR. HIGGINS: I SO MOVE.
DR. PRIETO: SO I'LL TAKE THAT AS A MOTION AND A SECOND FOR 09610.
MR. TORRES: CORRECT.

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DR. PRIETO: IS THERE ANY DISCUSSION?

DR. HIGGINS: I'D LIKE TO MAKE A VERY SHORT, QUICK COMMENT.

DR. PRIETO: GO AHEAD.

DR. HIGGINS: THE CRITICISM FOR THIS PROPOSAL WAS NOT KNOWING EXACTLY WHAT LEVEL OF ALPHA-SYNUCLEIN WAS REQUIRED FOR A CELL TO BECOME SORT OF A DISEASE CELL OR A TERMINAL CELL TARGETED FOR CELL DEATH. I WOULD ACKNOWLEDGE THAT, BUT I WOULD ALSO SAY THAT THE USE OF CRISPR-CAS9 BRINGS THE PARKINSON'S RESEARCH COMMUNITY SORT OF IN LINE WITH THE STATE-OF-THE-ART TECHNOLOGY AND WILL NOW GET PEOPLE THINKING MORE SO IN THOSE TERMS. I THINK I JUST WOULD ENCOURAGE EVERYONE TO SUPPORT THIS RESEARCH.

DR. PRIETO: THANK YOU, DAVID. ANY OTHER COMMENTS? CALL THE ROLL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: LET ME THINK ABOUT IT. NO. YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: JOE PANETTA.

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MR. PANETTA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: ABSTAIN.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: LAUREN, ARE YOU BACK ON THE LINE?
MOTION CARRIES.
DR. PRIETO: OKAY. SO WE'RE NOW AT 18.3 MILLION. I'D LIKE TO HEAR A MOTION FOR THE NEXT APPLICATION, 09631.
MR. TORRES: MR. CHAIRMAN, WE HAVE THREE MILLION REMAINING, AND THE LAST FOUR OBVIOUSLY WOULD EXCEED THAT.
DR. JUELSGAARD: HOW MUCH DO WE SPECIFICALLY HAVE? WHAT'S THE EXACT DOLLAR AMOUNT?
MR. TORRES: 3.012315 REMAINING.

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DR. PRIETO: DO I HEAR A MOTION TO APPROVE ONE OF THESE APPLICATIONS? THE NEXT ONE IN ORDER IS 09631. WE WILL RUN OUT OF MONEY AT SOME POINT, BUT WE NEED A MOTION TO APPROVE AN APPLICATION IN ORDER TO PROCEED.

MR. TORRES: IF I MAY, OUT OF ORDER I'D LIKE TO MOVE THE CYSTIC FIBROSIS GRANT.

MS. WINOKUR: I AGREE.

DR. PRIETO: IS THERE A SECOND?

DR. JUELSGAARD: SECOND.

DR. PRIETO: DIANE?

MS. WINOKUR: YES.

MR. JUELSGAARD: AND STEVE.

MS. BONNEVILLE: AND STEVE.

DR. PRIETO: OKAY.

MS. BONNEVILLE: 9637?

MS. CHEUNG: YES.

DR. DULIEGE: JUST TO BE CLEAR, THAT MEANS THAT NONE OF THE REMAINDER, 31542 AND 460 WILL BE APPROVED. WE'LL HAVE TO MAKE A CHOICE BETWEEN THESE THREE; IS THAT RIGHT?

CHAIRMAN THOMAS: IF YOU APPROVE THIS PARTICULAR PROJECT, WE WILL NOT BE ABLE TO APPROVE ANY OF THE REMAINING THREE BECAUSE THEY WILL PUT YOU THROUGH THE LIMIT.

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DR. DULIEGE: WE CAN CHOOSE WHAT WE BELIEVE IS THE MOST MERITORIOUS APPLICATION TO BE FUNDED RIGHT AWAY; IS THAT RIGHT?

DR. PRIETO: YES. WE HAVE FOUR APPLICATIONS LEFT. IF WE FUNDED THE FIRST TWO, WE WOULD COME UP TO OUR LIMIT. IF WE FUND THIS ONE, WE WILL NOT HAVE ENOUGH REMAINING TO FUND ANOTHER APPLICATION. IF WE FUND THE LAST ONE, THE PACEMAKER ONE, I THINK THAT WE ALSO WILL NOT HAVE ENOUGH REMAINING TO FUND ANOTHER APPLICATION.

MR. ROWLETT: SENATOR TORRES HAS MADE A MOTION FOR US AND IT'S BEEN SECONDED, IS THAT CORRECT, REGARDING --

MS. BONNEVILLE: YES.

MR. ROWLETT: SO MY QUESTION FOR STAFF IS REGARDING THIS PROPOSAL. I HAVE JUST A QUESTION. I DON'T RECALL WHAT SOME OF THE CONCERNS WERE. IF YOU COULD RESUMMARIZE WHAT THE CONCERNS WERE RELATED TO THIS PROPOSAL AGAIN.

CHAIRMAN THOMAS: AL, I'D LIKE TO ADD TO THAT QUESTION TO DR. SAMBRANO HOW DID THE PUBLIC COMMENT OF THOSE TESTIFYING ADDRESS ANY OF THESE ISSUES, IF THEY DID?

DR. SAMBRANO: SO THIS IS GIL. I'LL JUST GO OVER. THIS IS, AGAIN, HIGHLIGHTING SOME OF THE

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CONCERNS THAT ARE LISTED IN THE SUMMARY DOCUMENT. THE CONCERNS RELATED TO THE CHALLENGING ASPECT OF CORRECTING THE CFTR GENE SIMPLY BECAUSE IT HAS NOT YET BEEN ACHIEVED, ALTHOUGH THE APPLICANTS DO PROPOSE THAT THIS IS SOMETHING THAT THEY HAVE THE ABILITY TO DO. THAT A DECISION TO NOT INCLUDE A METHOD FOR PURIFYING OR ENRICHING FOR THE SUCCESSFULLY GENE-CORRECTED BASAL CELLS IS A LIMITATION. THAT THERE'S AN INSUFFICIENT FOCUS ON CELL TYPES THAT ARE SUCCESSFULLY GENE CORRECTED AND HAVE BOTH THE PROCESS OF GENE EDITING AND CONTINUED CULTURE OF THE CELLS AND PHENOTYPE AND SUITABILITY FOR TRANSPLANTATION. ACHIEVING THE PROJECT WITHIN A TWO-YEAR TIMELINE GIVEN THE AMBITIOUS NATURE OF THE PROJECT. THERE WAS SOME QUESTION ABOUT THE RATIONALE FOR TAKING A DUAL APPROACH OF CORRECTING A SINGLE MUTATION VERSUS WHAT THEY CALL THE UNIVERSAL STRATEGY IN WHICH THE FULL GENE IS REPLACED. AND THEN THE OTHER WAS RELATED TO THE CHOICE OF THE SINUSES AS THE IMPLANTATION SITE AND THE QUESTION OF WHETHER IT WOULD BE AS INFORMATIVE AS PLACEMENT INTO THE LUNG. SO THOSE WERE SOME OF THE CONCERNS.

NOW, WHETHER THE APPLICANT ADEQUATELY ADDRESSED THOSE CONCERNS IS SOMETHING I CANNOT ADDRESS. IT REALLY IS THE EXPERT OPINION OF THE GWG

PANEL, AND THE CONCERNS COME FROM THEM. SO I CANNOT SPEAK ON THEIR BEHALF IN TERMS OF WHETHER IT ADEQUATELY ADDRESSES THOSE.

DR. PRIETO: WOULD ANY OF THE PATIENT ADVOCATES WHO TOOK PART IN THE GWG DISCUSSION WANT TO RESPOND TO THAT?

MR. TORRES: YES. THE TESTIMONY WE HEARD TODAY WAS THAT THE PLACEMENT IS THE MOST APPROPRIATE PLACE, WHICH IS IN THE SINO CAVITY, TO DEAL WITH THIS ISSUE. AND THAT WAS RAISED ALSO AT OUR REVIEW. BUT IT STILL CAME UP WITH AN 85, WHICH IS STILL A PRETTY HIGH SCORE GIVEN THOSE CONCERNS THAT WERE RAISED. WHAT I HEARD TODAY, I THINK YOU HEARD IT AS WELL FROM THE EXPERT THAT TESTIFIED, WAS THAT THAT IS THE APPROPRIATE PLACEMENT.

DR. PRIETO: THANK YOU. ANY FURTHER DISCUSSION?

DR. DULIEGE: IF I CAN JUST MAKE A COMMENT. I REALIZE THE CHALLENGE THAT WE FACE. I WAS WONDERING IF THE CIRM STAFF CAN MAKE A RECOMMENDATION TO THE ICOC ON WHICH OF THESE FOUR APPEARS TO BE THE MORE MERITORIOUS RIGHT NOW. IF WE ARE GOING TO VOTE AS AN ICOC, I BELIEVE THAT, INCLUDING MYSELF, WE HAVE NO -- NOT THE RIGHT INFORMATION TO DIFFERENTIATE BETWEEN THESE FOUR.

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AND WE'RE GOING TO GO BY OUR OWN SENSITIVITY WHETHER WE LIKE MOST CYSTIC FIBROSIS VERSUS TRAUMATIC BRAIN INJURY, BUT I DON'T THINK IT'S NECESSARILY THE BEST WAY TO ADDRESS THAT. SO EITHER NOW OR SOON, IS THERE ANY OPTION TO HAVE A RECOMMENDATION FROM CIRM?

DR. PRIETO: CAN I COMMENT ON THAT?

MS. BONNEVILLE: YES.

DR. PRIETO: I'M NOT SURE THAT CIRM STAFF CAN OR WOULD WANT TO, BUT I WOULD POINT OUT THAT, IN TERMS OF THE MEDIAN SCORE, THESE FOUR ARE ALL EQUALLY MERITORIOUS. THEY WERE ALL CONSIDERED TO BE VERY GOOD SCIENCE AND WORTHY OF FUNDING. SO THE DECISION REALLY IS OURS, AND A BIG PART OF THAT DECISION DOES HAVE TO FALL DOWN TO OUR PROGRAMMATIC CONSIDERATIONS. SO THAT'S A VALID BASIS FOR MAKING A JUDGMENT. EACH ONE OF THESE HAS SOME, I DON'T KNOW IF I WANT TO SAY FLAWS, BUT QUESTIONS ABOUT THEIR VIABILITY, BUT I THINK THAT'S TRUE OF ANY APPLICATION.

CHAIRMAN THOMAS: FRANCISCO, RANDY WOULD LIKE TO MAKE A COMMENT.

DR. MILLS: JUST ONE COMMENT. JUST BECAUSE THEY'RE IN THE ROOM, THEY CAN MAKE A RECOMMENDATION. THEY'RE AN INCREDIBLY CAPABLE GROUP OF PEOPLE. WE DON'T, THOUGH, AS PART OF THE

PROCESS. AND SO WE WANT THAT TO BE DONE IN LIGHT OF THE GWG'S RECOMMENDATIONS AND THE BOARD'S DECISION.

CHAIRMAN THOMAS: FRANCISCO, I'D JUST LIKE TO MAKE A COMMENT THAT THIS IS, IN TERMS OF A CLASSIC PROGRAMMATIC REVIEW POINT, THE FACT THAT WE DON'T HAVE ANY PROJECTS IN THE PORTFOLIO FOR THIS PARTICULAR CONDITION IS SOMETHING THAT I WOULD ARGUE SHOULD WEIGH STRONGLY ON A POSITIVE CONSIDERATION OF THIS PROPOSAL.

DR. PRIETO: THANK YOU. ANY OTHER COMMENT?

CHAIRMAN THOMAS: FRANCISCO, THE OTHER POINT I'D LIKE TO MAKE, NOT ON THIS SPECIFIC PROPOSAL, BUT JUST AS A GENERAL MATTER IS TO REITERATE WHAT DR. SAMBRANO SAID EARLIER. IF THERE ARE PROJECTS THAT EITHER WERE APPROVED FOR FUNDING AND DON'T GET AN AWARD TODAY OR WERE NOT APPROVED FOR FUNDING AND WISH TO REAPPLY, IN EITHER INSTANCE, THERE WILL BE AN IMMEDIATE OPPORTUNITY TO DO THAT AS SOON AS FEBRUARY 15TH.

DR. PRIETO: THANK YOU. ANY FURTHER COMMENTS? HEARING NONE, CALL THE ROLL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: I HEAR THE QUESTION IS WHETHER WE APPROVE WHAT MOTION EXACTLY? CAN YOU

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JUST REPEAT IT AGAIN?

MR. TORRES: THE MOTION THAT I MADE WAS TO APPROVE --

MR. TOCHER: THE MOTION IS TO APPROVE APPLICATION 9637.

DR. DULIEGE: I APPROVE. YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: YES.

MS. BONNEVILLE: JOE PANETTA. FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: NO.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: YES.

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MS. BONNEVILLE: MOTION CARRIES.

DR. PRIETO: SO ANY FURTHER COMMENTS BEFORE WE CLOSE OUT THE MEETING?

MR. TOCHER: DR. PRIETO, WE HAVE ONE MORE MOTION I BELIEVE YOU WILL BE CALLING.

DR. PRIETO: CAN I HEAR A MOTION TO NOT FUND THE REMAINING APPLICATIONS?

DR. JUELSGAARD: SO MOVED.

MR. ROWLETT: SECOND.

DR. PRIETO: THANK YOU. DISCUSSION? CALL THE ROLL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: YES.

MS. BONNEVILLE: JOE PANETTA. FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: ABSTAIN.

MS. BONNEVILLE: AL ROWLETT.

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MR. ROWLETT: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MR. TOCHER: OS STEWARD, YOU CAN VOTE AYE OR NAY EXCEPT WITH RESPECT TO THOSE APPLICATIONS WITH WHICH YOU HAVE A CONFLICT.
DR. STEWARD: YES, EXCEPT FOR THOSE WITH WHICH I HAVE A CONFLICT.
MR. TOCHER: AND I'LL CONFIRM THAT YOU VOTED IN A SIMILAR MANNER ON THE FIRST MOTION REGARDING TIER II?
DR. STEWARD: YES, CORRECT.
MR. TOCHER: IS JOE PANETTA ON THE LINE?
MS. BONNEVILLE: JUST ONE SECOND PLEASE. WE JUST NEED TO GET JOE BACK ON THE PHONE.
CHAIRMAN THOMAS: FRANCISCO, J.T. FOR PROCEDURAL PURPOSES, ONCE WE GET THROUGH THIS, THIS WILL CONCLUDE THE PROGRAMMATIC REVIEW CONSIDERATION OF THE APPLICATIONS. WE STILL HAVE GENERAL PUBLIC COMMENT ON THE AGENDA TO GO --
DR. PRIETO: YES. THANK YOU.

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CHAIRMAN THOMAS: -- SO IT WON'T QUITE CONCLUDE THE MEETING.

MS. BONNEVILLE: WE'RE GOOD.

MR. TOCHER: THE MOTION CARRIES.

CHAIRMAN THOMAS: SO, DR. PRIETO, THANK YOU VERY MUCH FOR A VERY WELL-RUN PROGRAMMATIC REVIEW OF THESE ITEMS. THAT CONCLUDES THE PROGRAMMATIC REVIEW SESSION.

THE ONLY ITEM WE HAVE REMAINING, IS THERE ANY PUBLIC COMMENT ON ANY MATTERS OF ANY SORT THAT ANYONE WOULD LIKE TO MAKE EITHER HERE OR ON THE PHONE?

MS. BONNEVILLE: I JUST WANTED TO REMIND ALL OF OUR BOARD MEMBERS THAT WE HAVE AN IN-PERSON BOARD MEETING ON FEBRUARY 23D, AND WE WILL SEND OUT DETAILS SHORTLY. WE WILL BE HAVING IT HERE AT CIRM HEADQUARTERS.

CHAIRMAN THOMAS: THANK YOU, EVERYBODY, FOR YOUR PARTICIPATION. WE STAND ADJOURNED.

DR. PRIETO: THANK YOU.

(THE MEETING WAS THEN CONCLUDED AT 11:51 A.M.)

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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 TELEPHONIC 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 19, 2017, 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, CSR 7152
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