BEFORE THE
SCIENCE SUBCOMMITTEE AND NEURO TASK FORCE ON
NEUROSCIENCE AND MEDICINE

OF THE INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE TO THE

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE ORGANIZED PURSUANT TO THE CALIFORNIA STEM CELL RESEARCH AND CURES ACT

**REGULAR MEETING** 

LOCATION: VIA ZOOM

DATE: JULY 11, 2024

9 A.M.

REPORTER: BETH C. DRAIN, CA CSR

CSR. NO. 7152

FILE NO.: 2024-30

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OPEN SESSION	
1. CALL TO ORDER	3
2. ROLL CALL	3
3. REVIEW STRATEGIC ALLOCATION FRAMEWORK GOALS 1 AND 2 AND POTENTIAL RECOMMENDATIONS	5
4. PUBLIC COMMENT	NONE
5. ADJOURNMENT	60

1	JULY 11, 2024; 9 A.M.
2	
3	CHAIRMAN FISCHER-COLBRIE: OKAY. GREAT.
4	WELL, WELCOME TO THE JOINT MEETING OF THE NEURO TASK
5	FORCE AND THE SCIENCE SUBCOMMITTEE. AND WE'RE VERY
6	PLEASED TO CONTINUE THE DISCUSSION RELATED TO THE
7	STRATEGIC ALLOCATION FRAMEWORK AND THE OTHER AGENDA
8	ITEMS. BUT WITH THAT IN MIND, FIRST, IF WE COULD
9	HAVE A ROLL CALL. SCOTT, IF YOU COULD LEAD US IN A
10	ROLL CALL.
11	MR. TOCHER: SURE.
12	MARIA BONNEVILLE.
13	VICE CHAIR BONNEVILLE: PRESENT.
14	MR. TOCHER: LEONDRA CLARK-HARVEY.
15	DR. CLARK-HARVEY: PRESENT.
16	MR. TOCHER: DEBORAH DEAS. MARK
17	FISCHER-COLBRIE.
18	CHAIRMAN FISCHER-COLBRIE: PRESENT.
19	MR. TOCHER: FRED FISHER. ELENA FLOWERS.
20	JUDY GASSON.
21	CHAIRPERSON GASSON: HERE.
22	MR. TOCHER: DAVID HIGGINS.
23	DR. HIGGINS: PRESENT.
24	MR. TOCHER: VITO IMBASCIANI.
25	CHAIRMAN IMBASCIANI: HERE.
	3

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	2211 0.211111, 0.1 001110 201
1	MR. TOCHER: PAT LEVITT.
2	DR. LEVITT: HERE.
3	MR. TOCHER: SHLOMO MELMED.
4	DR. MELMED: HERE.
5	MR. TOCHER: CAROLYN MELTZER.
6	DR. MELTZER: PRESENT.
7	MR. TOCHER: LAUREN MILLER-ROGEN. CHRIS
8	MIASKOWSKI.
9	DR. MIASKOWSKI: PRESENT.
10	MR. TOCHER: MARV SOUTHARD.
11	DR. SOUTHARD: PRESENT.
12	MR. TOCHER: KAROL WATSON.
13	DR. WATSON: HERE.
14	MR. TOCHER: KEITH YAMAMOTO.
15	DR. YAMAMOTO: HERE.
16	MR. TOCHER: THANK YOU VERY MUCH, KEITH.
17	GREAT. WE'RE READY TO GO. THANKS VERY MUCH, MARK.
18	CHAIRMAN FISCHER-COLBRIE: GREAT. WELL,
19	BEFORE WE KICK OFF, FIRST OF ALL, I'D LIKE TO HAVE
20	OUR NEWLY APPOINTED CEO, JON THOMAS, MAKE SOME
21	INTRODUCTORY COMMENTS BEFORE WE CONTINUE WITH THE
22	FORMAL AGENDA.
23	SO, J.T., I'M VERY EXCITED ABOUT YOUR
24	APPOINTMENT, BUT WOULD LOVE TO HAVE YOU GIVE
25	COMMENTS HERE.

1	DR. THOMAS: THANK YOU VERY MUCH, MARK.
2	THANK YOU, ALL MEMBERS OF THE BOARD. IT'S A
3	PLEASURE TO BE HERE IN THE INITIAL MEETING IN THIS
4	CAPACITY OF OUR VERY MAJOR LIFT HERE THAT WE'VE BEEN
5	DOING NOW FOR SIX MONTHS. WE'RE ABOUT TWO-THIRDS OF
6	THE WAY THROUGH. AND IT'S GETTING INTO NOW THE MEAT
7	OF RECOMMENDATIONS TO DISCUSS WITH ALL OF YOU.
8	I WANT TO SAY WE HAVE THE PLEASURE OF
9	BEING JOINED IN OUR OFFICES BY MR. JENSEN, WHO IS
10	OVER ON THE OTHER SIDE OF THE ROOM AND IS KEENLY
11	INTERESTED IN EVERYTHING WE'RE TALKING ABOUT HERE AS
12	WELL HE SHOULD BE. IT'S REALLY GOOD STUFF.
13	SO WITH THAT, I WANT TO TURN IT OVER TO
14	ROSA FOR HER REPORT ON WHERE WE STAND CURRENTLY IN
15	THE PROCESS. SO, ROSA, THANK YOU VERY MUCH.
16	DR. CANET-AVILES: THANK YOU, J.T. AND
17	THANK YOU, SARA, FOR RUNNING THE SLIDES. CAN YOU
18	ALL HEAR ME?
19	MR. TOCHER: YES.
20	DR. CANET-AVILES: WONDERFUL. SO MR.
21	CHAIRMAN OF THE SCIENCE SUBCOMMITTEE AND MADAM
22	CO-CHAIR AND MR. CO-CHAIR OF THE NEURO TASK FORCE,
23	ON BEHALF OF CIRM TODAY, I WILL BE PRESENTING THE
24	NEXT STEPS IN THE ANALYSIS, THE RESULTS OF THE
25	ANALYSIS FOR THE RECOMMENDATIONS AND THE
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1	RECOMMENDATIONS FOR DISCUSSION. NEXT SLIDE OF THE
2	FIRST TWO GOALS IN OUR STRATEGIC ALLOCATION
3	FRAMEWORK.
4	THANK YOU. SO TODAY, IN ORDER TO ENSURE
5	AMPLE TIME FOR DISCUSSION, THE BACKGROUND AND THE
6	STRATEGIC ALLOCATION FRAMEWORK OVERVIEW WILL NOT BE
7	PRESENTED DURING TODAY'S MEETING. WE ADDED A
8	REFERENCE TO WHEN THESE SECTIONS WERE PREVIOUSLY
9	PRESENTED AT THE JUNE 27TH ICOC MEETING AND ALSO
10	PRESENTED AT THE PREVIOUS SCIENCE SUBCOMMITTEE/NEURO
11	TASK FORCE JOINT MEETING. SO IF YOU ARE INTERESTED
12	OR YOU HAVEN'T HAD TIME TO ADDRESS THIS, YOU CAN
13	REFER TO THAT LINK THAT WILL LEAD YOU DIRECTLY TO
14	THE MOMENT IN THE VIDEO WHEN WE STARTED PRESENTING.
15	NEXT SLIDE.
16	SO TODAY'S PRESENTATION WILL FOCUS ON
17	GOALS 1 AND 2 THAT WERE INTRODUCED FOR THE FIRST
18	TIME AT THE LAST NEURO TASK FORCE/SCIENCE
19	SUBCOMMITTEE JOINT MEETING AND SUBSEQUENTLY AT THE
20	ICOC OF JUNE.
21	THE PRESENTATION SHOULD TAKE ABOUT HALF OF
22	OUR MEETING MAXIMUM. IF I CAN, I WILL TRY TO GO
23	FASTER. AND THE OTHER HALF SHOULD BE FOR
24	DISCUSSION. NEXT SLIDE.
25	I'M GOING TO TAKE A MOMENT HERE TO GO OVER

1	THE PROCESS HOW WE DEVELOPED THESE GOALS. THE GOALS
2	WERE DEVELOPED THROUGH A SERIES OF STRATEGIC
3	PLANNING DISCUSSIONS THAT WERE STRUCTURED AROUND
4	ALIGNING WITH THE OVERARCHING STRATEGIC PLAN THAT WE
5	HAVE HAD SINCE 2020 WHILE REMAINING ADAPTABLE TO
6	EMERGING SCIENTIFIC, TECHNOLOGICAL, AND OTHER
7	OPPORTUNITIES LIKE MARKET OPPORTUNITIES.
8	KEY ELEMENTS OF THE PROCESS WERE ALREADY
9	TALKED ABOUT, LIKE WE THOUGHT ABOUT IMPACT
10	POTENTIAL, PATIENT REACH, TECHNOLOGY, PROSPECTS FOR
11	REGULATORY APPROVAL, BUT THEY ALSO INCLUDED THINGS
12	LIKE STRATEGIC HORIZON MAPPING, SO EVALUATING THE
13	CHALLENGES AND OPPORTUNITIES FOR CIRM'S PATH FORWARD
14	IN THE NEXT MORE OR LESS NEXT DECADE. THE
15	PRACTICALITY OF THESE IMPACT GOALS, THE IMPACT GOALS
16	VERSUS THE CONSTRAINTS IN PRACTICALITY. FLEXIBILITY
17	AS WELL, EMPHASIZING THE IMPORTANCE OF ADAPTING AND
18	PLANNING TO ACCOMMODATE NEW OPPORTUNITIES AND
19	CHALLENGES. BUT ALSO AN IMPORTANT PART WAS
20	ADDRESSING A DIVERSE DISEASE SPECTRUM.
21	AS A GOVERNMENT AGENCY, ONE MAJOR
22	CHALLENGE IS THE WIDE RANGE OF DISEASES FROM RARE TO
23	COMMON. AND EACH OF THEM REQUIRES A SPECIFIC
24	RESEARCH FOCUS AND RESOURCES.
25	HISTORICALLY OUR EFFORTS HAVE

1	PREDOMINANTLY TARGETED RARE DISEASES, WHICH HAS
2	ALLOWED US TO MAKE SIGNIFICANT STRIDES IN AREAS THAT
3	OFTEN LACK ATTENTION AND FUNDING. BY CONCENTRATING
4	ON THESE CONDITIONS, CIRM HAS CATALYZED ADVANCEMENTS
5	IN THE TRANSLATION OF THIS FUNDING INTO CLINICAL
6	APPLICATIONS EVEN FOR SOME DISEASES, LIKE SICKLE
7	CELL, THAT ARE THRESHOLD, THE PREVALENT, RIGHT.
8	WHEN LOOKING AT OUR HISTORY AND PORTFOLIO, ONE OF
9	THE CONSIDERATIONS THAT CIRM HAS NOT MADE WAS HOW
10	COULD CIRM MAKE AN IMPACT TO DISEASES THAT AFFECT
11	MOST CALIFORNIANS.
12	SO BY EXTENDING OUR FOCUS TO INCLUDE
13	PREVALENT DISEASES ALONGSIDE RARE ONES, WE HOPE THAT
14	WE WILL NOT ONLY BROADEN OUR IMPACT, BUT ALSO
15	DEMONSTRATE OUR COMMITMENT TO IMPROVING THE HEALTH
16	OUTCOMES FOR ALL CALIFORNIANS.
17	IMPORTANTLY, THIS EXPANSION DOES NOT MEAN
18	THAT CIRM WILL CEASE FUNDING IN RARE DISEASES. ON
19	THE CONTRARY SINCE CIRM IS ACTIVELY DEVELOPING A
20	STRATEGY UNDER DR. CREASEY'S LEADERSHIP TO OPTIMIZE
21	OUR INVESTMENTS IN RARE DISEASES. AND THIS
22	STRATEGIC APPROACH WILL INITIALLY BRING A HIGH LEVEL
23	OVERVIEW OF WHAT THIS WILL BE ABOUT DURING THE NEXT
24	MEETING SCHEDULED FOR AUGUST 16TH WHERE WE WILL BE
25	TALKING ABOUT OR PRESENTING THE RECOMMENDATIONS FOR

1	GOALS 3 AND 4 RESPECTIVELY.
2	SO I WANT TO ENSURE THAT WE ARE ALL
3	ALIGNED AND UNDERSTAND THE DUAL FOCUS OF OUR
4	STRATEGIC EXPANSION.
5	SO THROUGH THE DATA THAT WE'VE GATHERED
6	AND THE STRATEGIC ALLOCATION FRAMEWORK EXERCISE, WE
7	AIM TO INTEGRATE NEW SCIENTIFIC INSIGHTS WITH OUR
8	PROVEN APPROACHES TO DISCOVERY AND TRANSLATION,
9	ENSURING THAT EVERY CALIFORNIAN BENEFITS FROM THE
10	ADVANCEMENTS IN STEM CELL AND GENETIC RESEARCH.
11	NOW, FOR PREVALENT DISEASES, IT'S VERY
12	IMPORTANT TO SUPPORT EARLY STAGE RESEARCH WHERE
13	TRADITIONAL VENTURE CAPITAL AND INDUSTRY FUNDING ARE
14	MORE CAUTIOUS, BUT PARTICULARLY IN OUR CURRENT
15	ECONOMIC CONDITIONS AS WELL. SO WE, AS A FUNDING
16	AGENCY OF THE STATE OF CALIFORNIA, HAVE A
17	RESPONSIBILITY TO EVALUATE THE TYPE OF ROLE THAT WE
18	CAN PLAY IN THOSE EARLY STAGES. AND TODAY IS ONE OF
19	THE DAYS THAT WE WILL BE DISCUSSING THIS BECAUSE IT
20	APPLIES MORE TO THE DISCOVERY AREA OF CIRM'S
21	FUNDING. NEXT SLIDE. THANK YOU.
22	SO TODAY'S PRESENTATION, THE GOAL OF TODAY
23	IS TO BASICALLY REVIEW THE PRELIMINARY GOALS OF 1
24	AND 2 AND THE HIGH LEVEL QUESTIONS THAT WE POSED,
25	THE DATA ANALYSIS, THE RECOMMENDATIONS, AND THEN
	_

1	HAVE A DISCUSSION. SO LET'S GO INTO THE FIRST GOAL.
2	AS I WAS SAYING, IT IS IMPORTANT TO
3	SUPPORT EARLY STAGE RESEARCH WHERE TRADITIONAL
4	VENTURE CAPITAL AND INDUSTRY ARE MORE CAUTIOUS. AND
5	IN ORDER TO DO THAT, THE FIRST GOAL, WHICH WAS OUR
6	WORKING HYPOTHESIS, WAS DEFINED AS CATALYZING THE
7	IDENTIFICATION AND VALIDATION OF AT LEAST X NOVEL
8	TARGETS AND BIOMARKERS, ENSURING INTEGRATION INTO
9	PRECLINICAL OR CLINICAL RESEARCH FOR DISEASES IN
10	CALIFORNIA.
11	WE ADDED AN X. WE DIDN'T WANT TO
12	DELINEATE THE NUMBER BECAUSE WE THOUGHT THAT THE
13	BOARD MIGHT WANT TO HAVE A DISCUSSION AROUND THAT.
14	WE DIDN'T WANT TO SAY THREE, FOUR, FIVE. WE CAN
15	DISCUSS THIS DURING THE RECOMMENDATION, THE
16	DISCUSSION TIME.
17	NOW, IN ORDER TO DELINEATE TO FIGURE
18	OUT WHAT THE RECOMMENDATIONS COULD BE, WE ASKED THE
19	FOLLOWING QUESTIONS AT A VERY HIGH LEVEL. WITHIN
20	PORTFOLIO SCOPE AND DISEASE REPRESENTATION, WE ASKED
21	OURSELVES WHICH DISEASES IN CALIFORNIA COULD BENEFIT
22	MOST FROM IDENTIFICATION AND VALIDATION OF NOVEL
23	TARGETS AND BIOMARKERS? AND WHAT DOES THE DISEASE
24	BURDEN AND PREVALENCE DATA INDICATE ABOUT PRIORITY
25	HEALTH OUTCOMES IN OUR STATE? WHICH OF THESE ARE

1	MORE AMENABLE TO DISCOVERY OF TARGETS/BIOMARKERS
2	UTILIZING STEM CELL AND/OR GENETIC RESEARCH?
3	SO ALL OF THIS IS WHAT WE ARE TRYING TO DO
4	TODAY IN A VERY SHORT MEETING IS PRESENT HOW THE
5	DATA LED TO THE RECOMMENDATIONS THAT WE WILL
6	PRESENT.
7	IN TERMS OF COLLABORATION, HOW CAN WE
8	LEVERAGE AND INCENTIVIZE MULTIPLE STAKEHOLDER
9	COLLABORATION TO ACCELERATE THE DISCOVERY AND
10	VALIDATION OF NOVEL TARGETS AND BIOMARKERS?
11	AND INNOVATION AND TECHNOLOGY, WHAT NEW
12	TECHNOLOGIES AND RESEARCH METHODS COULD ADVANCE THE
13	DISCOVERY AND VALIDATION OF NOVEL TARGETS AND
14	BIOMARKERS? NOW, THIS SLIDE IS VERY CONCISE. IT
15	TOOK A LOT OF DAYS AND LONG DISCUSSIONS AT THE LEVEL
16	OF THE LEADERSHIP TEAM AND DISCUSSING WITH THE
17	SCIENCE TEAM LEADS AS WELL TO GET TO THESE SUCCINCT
18	QUESTIONS. WE WANT TO STREAMLINE THIS APPROACH. SO
19	THIS SEEMS SIMPLE, BUT WE HAD A LOT A LOT OF
20	THOUGHT WENT INTO PUTTING TOGETHER THESE QUESTIONS.
21	THE NEXT GOAL IS ACCELERATE THE
22	DEVELOPMENT AND UTILIZATION OF X TECHNOLOGIES THAT
23	DEMONSTRATE IMPROVEMENT IN SAFETY, EFFICACY, AND
24	QUALITY OF CELL AND GENE THERAPIES. AS WE ALL KNOW,
25	IN ORDER TO ACHIEVE BROAD APPLICABILITY OF CELL AND

1	GENE THERAPIES FOR RARE AND PREVALENT DISEASES,
2	THERE'S A NEED TO IMPLEMENT TECHNOLOGY PLATFORMS
3	THAT CAN ENSURE THE SAFETY, EFFICACY, AND
4	RELIABILITY OF MULTIPLE CELL AND GENE THERAPIES.
5	AND THIS WAS THE FRAMING FOR THE DESIGN OF THE
6	SECOND GOAL WITH A FOCUS ON BRIDGING THE GAP BETWEEN
7	CUTTING-EDGE ACADEMIC RESEARCH IN CELL AND GENE
8	THERAPIES AND INDUSTRY AND ITS COMMERCIALIZATION.
9	SO THE HIGH LEVEL QUESTIONS THAT WE AS A
10	TEAM DEVELOPED WERE CURRENT DEVELOPMENT BOTTLENECKS.
11	WHAT ARE THE CURRENT TRANSLATIONAL BOTTLENECKS FOR
12	CELL AND GENE THERAPIES? WHAT IS THE INDUSTRY
13	LANDSCAPE? WHAT IS INDUSTRY LACKING THAT WE COULD
14	INVEST TO MAKE ACADEMIA AND INDUSTRY BY
15	COLLABORATING ACCELERATE THAT? RIGHT.
16	INNOVATION AND TECHNOLOGY, WHAT INNOVATIVE
17	TECHNOLOGIES AND RESEARCH METHODOLOGIES COULD BE
18	UTILIZED OR DEVELOPED TO ADDRESS
19	DEVELOPMENT/TRANSLATIONAL BOTTLENECKS?
20	INFRASTRUCTURE UTILIZATION, HOW WILL
21	CLINICAL, MANUFACTURING, AND PATIENT SUPPORT
22	INFRASTRUCTURES THAT WE ARE ALREADY IMPLEMENTING BE
23	OPTIMIZED TO SUPPORT THESE OBJECTIVES? ALL OUR
24	PROGRAMS ARE WORKING IN COGWHEEL. EVERYTHING IS
25	INTERACTIVE. SO WE NEED TO FIGURE OUT HOW ARE WE

1	GOING TO LEVERAGE INTERNALLY.
2	AND THEN ALSO EXTERNALLY, FOSTERING
3	COLLABORATION. HOW CAN CIRM FOSTER COLLABORATION
4	BETWEEN ACADEMIC AND INDUSTRY STAKEHOLDERS TO
5	ADVANCE DEVELOPMENT AND UTILIZATION OF THE NOVEL
6	TECHNOLOGIES? NEXT SLIDE.
7	NOW, THIS SLIDE IS GOING OVER THE DATA
8	SOURCES. OUR ANALYSIS AND RECOMMENDATIONS HAVE BEEN
9	GUIDED BY A VERY ROBUST, COMPREHENSIVE DATASET. THE
10	APPROACH HAS BEEN BOTH COMPREHENSIVE AND METICULOUS,
11	ALSO TRYING NOT TO GO DOWN THE RABBIT HOLE BECAUSE
12	THERE'S ALWAYS A LOT OF DATA. ANYWHERE YOU LOOK,
13	YOU HAVE TO BE PRECISE. AND THAT'S WHY THE
14	QUESTIONS WERE VERY IMPORTANT. AND WE ENSURED THAT
15	EVERY STRATEGIC COLLABORATION WAS BACKED BY SOLID
16	DATA AND REAL-WORLD INSIGHT.
17	SO THIS PAGE SHOWS THE MAIN RESOURCES OF
18	DATA THAT WE HAVE CONSULTED INTERNALLY AND
19	EXTERNALLY. SO WE CONSULTED DATA FROM THE
20	CALIFORNIA DEPARTMENT OF PUBLIC HEALTH REPORTS, THE
21	CDC, AND THE CANCER REGISTRY REPORTS. THESE DATA
22	HERE INCLUDED SEVERAL YEARS LEADING UP TO 2023 AND
23	EARLY 2024 AND COVERED BOTH PREPANDEMIC AND PANDEMIC
24	PERIODS. THIS TIME FRAME ALLOWED FOR A
25	COMPREHENSIVE ANALYSIS THAT ACCOUNTS FOR POTENTIAL

1	ABERRATIONS CAUSED BY UNUSUAL EVENTS SUCH AS
2	COVID-19, FOR EXAMPLE. WE TRIED TO TAKE A SNAPSHOT
3	WITH A MULTIYEAR APPROACH TO UNDERSTAND BROADER
4	TRENDS IN HEALTH DATA IN CALIFORNIA, WHICH ALLOWS US
5	TO ALIGN OUR STRATEGIES WITH THE CURRENT HEALTH
6	LANDSCAPE OF CALIFORNIA.
7	THE SECOND TYPE OF DATA WAS OUR INTERNAL
8	PORTFOLIO DATA ANALYSIS. BY EXAMINING OUR OWN
9	HISTORICAL DATA, WE GAIN INSIGHTS INTO THE OUTCOMES
10	AND EFFECTIVENESS OF PAST PROJECTS, WHICH IS
11	INVALUABLE FOR FUTURE PROJECT SELECTION AND FUNDING
12	DISTRIBUTION ALLOCATION AND FOCUS.
13	A VERY IMPORTANT PART OF OUR DATA WAS
14	INDEPENDENT RESEARCH BY PROJECT LEADS AND SCIENCE
15	OFFICERS. WE HAVE A VERY DEDICATED TEAM OF PROJECT
16	LEADS AND SCIENCE OFFICERS THAT UNDERTOOK A DEEP
17	DIVE INTO THE DIFFERENT ASPECTS OF OUR PORTFOLIO AND
18	LANDSCAPE ANALYSIS CAPTURED THROUGH DATABASES AS
19	WELL AS THROUGH PEER REVIEW PAPERS AND RESEARCH
20	ARTICLES. SOME OF THE DATA THAT WE GATHERED IS NOT
21	FOUND IN A REPORT OR A DATABASE. YOU HAVE TO GO
22	THROUGH ARTICLES AND ALSO THROUGH YOUR EXPERTISE.
23	SO THAT WAS ESSENTIALLY EXTRACTED BY THE SCIENCE
24	TEAM AT CIRM.
25	THE FOCUS SIDE OF THE PORTFOLIO ANALYSIS

1	WAS ALSO ON CELL AND GENE THERAPY AMENABILITY,
2	BIOMARKER NEEDS, STEM CELL MODEL READINESS AND
3	NEEDS, AS WELL AS TECHNOLOGY AND BOTTLENECKS, GAPS.
4	BUT FOR TODAY'S DISCUSSION, THE FOCUS WILL NOT BE
5	CELL AND GENE THERAPY AMENABILITY. I WANT TO MAKE
6	SURE THAT IS CLEAR BECAUSE THIS IS COMING TO THE
7	AUGUST MEETING. TODAY'S FOCUS IS, AGAIN, TO FIGURE
8	OUT THE NEED FOR FURTHER DISCOVERY, BOTTLENECKS, AND
9	TECHNOLOGIES THAT WILL HELP US ADVANCE RESEARCH FOR
10	BOTH RARE AND PREVALENT DISEASES.
11	ANOTHER PART OF THE DATA THAT WE HAVE WAS
12	IQVIA'S CALIFORNIA DISEASE LANDSCAPE ANALYSIS OF
13	DISEASE STATES AFFECTING THE CALIFORNIA PATIENT
14	POPULATION. SO WE KIND OF LIKE WENT THROUGH TWO
15	APPROACHES TO THE CALIFORNIA PATIENT POPULATION, AND
16	IQVIA WAS ONE OF THEM. UTILIZING ANONYMIZED PATIENT
17	CLAIMS DATA FROM OVER 1.5 BILLION PATIENT
18	INTERACTIONS OVER THE PAST YEAR MATCHED TO ICD-10
19	MEDICAL CODES. THE ANALYSIS THAT IQVIA PROVIDED
20	BRINGS A DEEP UNDERSTANDING OF DISEASE PREVALENCE
21	AND MANAGEMENT TRENDS ACROSS CALIFORNIA. THE
22	INSIGHTS, WE ALSO GATHERED INSIGHTS FROM SUBJECT
23	MATTER EXPERTS AND HARVESTING ECONOMICS DATA WHICH
24	FURTHER REFINED OUR UNDERSTANDING OF WHERE STRATEGIC
25	INVESTMENT CAN BE MOST IMPACTFUL. THEY ALSO

1	GATHERED NIH FUNDING AND INDUSTRY LANDSCAPE DATA.
2	ANOTHER SOURCE OF FUNDING WAS THE
3	GLOBALDATA DATABASE THAT HAS PROVIDED US WITH A
4	BROADER INDUSTRY PERSPECTIVE. AND THAT INFORMATION
5	IS CRUCIAL TO ENSURE THAT OUR STRATEGY IS NOT ONLY
6	RESPONSIVE TO CURRENT NEEDS, BUT ALSO ANTICIPATORY
7	OF FUTURE SCIENTIFIC AND MARKET NEEDS AND SHIFTS.
8	AND ULTIMATELY WE ALREADY HEARD AT THE LAST NEURO
9	TASK FORCE/SCIENCE SUBCOMMITTEE THAT WE PRESENTED
10	THE SURVEY OF 670 NEUROSCIENTISTS ACROSS THE U.S.
11	MOSTLY THAT LED TO SOME PRELIMINARY RECOMMENDATIONS
12	THAT HAVE BEEN INCLUDED IN THE OVERALL GOALS AND
13	RECOMMENDATIONS THAT WE ARE PRESENTING TODAY.
14	SO TOGETHER THESE DATA SOURCES CREATE A
15	COMPREHENSIVE PICTURE THAT GUIDES THE STRATEGIC
16	ALLOCATION FRAMEWORK. AND AN IMPORTANT POINT TO
17	HIGHLIGHT IS THAT THE DATA THAT WE WILL BE SHOWING
18	HERE IS A SNAPSHOT REPRESENTATIVE OF ALL THE DATA
19	GATHERED THROUGH THESE DATA SOURCES WHICH COULD NOT
20	BE POSSIBLE TO SHOW IN A 1.5 HOUR OR EVEN TWO-HOUR
21	MEETING. SO NEXT SLIDE, SARA.
22	THIS SLIDE IS VERY IMPORTANT. THIS IS THE
23	PEOPLE THAT HAVE BEEN BEHIND THIS PRESENTATION. AS
24	I PRESENTED ON BEHALF OF CIRM AT THE BEGINNING BACK
25	IN MARCH DURING THE INITIAL STRATEGIC ALLOCATION

1	FRAMEWORK PRESENTATION, WE PROVIDED AN OVERVIEW OF
2	HOW THE LEADERSHIP TEAM AT CIRM HAD BEEN DEVELOPING
3	THE GOALS, THE QUESTIONS, AND DATA NEEDED IN ORDER
4	TO MAKE THE RECOMMENDATIONS. WE WERE STILL DRAFTING
5	THINGS, BUT THERE WAS A PROCESS THAT WAS LED THROUGH
6	THE LEADERSHIP TEAM. BUT THERE IS ANOTHER VERY,
7	VERY ESSENTIAL GROUP OF PEOPLE WHO HAVE BEEN WORKING
8	VERY HARD OVER THE PAST 2.5, 3 MONTHS GATHERING AND
9	ANALYZING A LOT OF DATA. AND THOSE PEOPLE ARE SHOWN
10	HERE.
11	OUR DEDICATED TEAM OF PROJECT LEADS AND
12	SCIENCE OFFICERS UNDERTOOK A DEEP DIVE INTO THE
13	DIFFERENT ASPECTS OF OUR PORTFOLIO AND LANDSCAPE
14	ANALYSIS, WHICH IS CAPTURED THROUGH DATABASES. BUT,
15	AS I WAS SAYING EARLIER ON, MUCH OF THE DATA AS WELL
16	IS IN PEER REVIEW PAPERS AND RESEARCH ARTICLES,
17	WHICH IS NOT FOUND IN REPORTS, AND NEEDS TO BE
18	EXTRACTED THROUGH LITERATURE AND EXPERT KNOWLEDGE.
19	SO I WANT TO THANK ALL OF THEM BECAUSE, WITHOUT
20	THEM, THIS COULD NOT HAVE BEEN POSSIBLE.
21	AND NOW, I WOULD LIKE TO MAKE A POINT TO
22	ACKNOWLEDGE THREE KEY PEOPLE. DR. SARA TAYLOR AND
23	THOMAS TRINH WITHOUT WHOM THE COORDINATION OF TEAM
24	MEMBERS ANALYSIS AND PUTTING TOGETHER THIS
25	PRESENTATION COULD NOT HAVE BEEN POSSIBLE. AND ALSO

1	SPECIALLY DR. SHYAM PATEL, WHO, BESIDES BEING A
2	MEMBER OF THE LEADERSHIP TEAM, LED AND COORDINATED
3	THE GLOBALDATA AND IQVIA EXTERNAL ANALYSIS EFFORTS
4	AND COORDINATED ALSO WITH SARA AND THOMAS. SO I
5	WANT TO THANK THEM. ALL THESE CONTRIBUTIONS WERE
6	ENGAGED IN CONJUNCTION WITH OUR REGULAR DUTIES,
7	UNDERSCORING THE DEDICATION AND HARD WORK OF OUR
8	TEAM. SO WE ARE PROFOUNDLY GRATEFUL FOR THEIR
9	COMMITMENT AND EXCELLENCE.
10	SO NOW LET'S GO TO THE FIRST SLIDE THAT
11	SHOWS THE DATA. THE NEXT FOUR SLIDES PRESENT A
12	SUMMARIZED SNAPSHOT OF OUR COMPREHENSIVE DATA
13	CRUCIAL FOR GUIDING THE STRATEGIC ALLOCATION
14	FRAMEWORK. I WOULD LIKE TO EMPHASIZE THAT THE
15	TABLES DISTILL KEY ELEMENTS FROM OUR BROADER DATASET
16	THAT HAS BEEN EXTENSIVELY GATHERED TO INFORM OUR
17	DECISION-MAKING PROCESS. WHILE THIS SUMMARY
18	PROVIDES VALUABLE INSIGHTS INTO OUR STRATEGIC
19	CONSIDERATIONS, PLEASE NOTE THAT IT REPRESENTS,
20	AGAIN, A SNAPSHOT OF IN-DEPTH ANALYSIS THAT WE HAVE
21	CONDUCTED.
22	AS I MENTIONED, IN ORDER TO ASSESS OUR
23	STRATEGIC FOCUS, WE FIRST TURNED OUR ATTENTION TO
24	THE MOST COMMON DISEASES AFFECTING CALIFORNIANS.
25	OUR ANALYSIS REVEALED A CRITICAL GAP IN OUR

1	PORTFOLIO, LACK OF BALANCED INVESTMENT IN CONDITIONS
2	THAT ARE NOT ONLY WIDESPREAD, BUT ALSO CARRY
3	SIGNIFICANT SOCIOECONOMIC AND DISEASE BURDENS FOR
4	THE STATE'S POPULATION.
5	THIS HIGH LEVEL SUMMARY TABLE HIGHLIGHTS
6	DISEASE-BASED PATIENT COUNTS INDICATING THE SCALE OF
7	IMPACT FOR EACH CONDITION. THE SUMMARY IS NOT MEANT
8	TO SHOW THE DISEASES THAT WE ARE PROPOSING. THIS IS
9	IMPORTANT TO FUND, BUT AN IDEA OF WHAT THE DISEASES
10	THAT ARE AFFECTING MOST CALIFORNIANS NEED IN ORDER
11	TO ADVANCE AND ACCELERATE THE DEVELOPMENT OF
12	THERAPIES. AGAIN, THIS IS NOT WHAT WE ARE TRYING TO
13	FUND. IT'S JUST TO GIVE US AN IDEA OF HOW CAN WE
14	INVEST IN EARLIER RESEARCH THAT WILL HAVE AN IMPACT
15	FOR PREVALENT DISEASES IN CALIFORNIA, NOT ONLY EARLY
16	RESEARCH, BUT, AS YOU WILL SEE, TECHNOLOGY PLATFORMS
17	AS WELL. SO HOPEFULLY THIS IS CLEAR.
18	FOR INSTANCE, THERE ARE OVER 4.4 MILLION
19	CALIFORNIANS LIVING WITH HYPERTENSION. IT IS MOSTLY
20	A COMORBIDITY OF OTHER DISEASES. AND NEARLY 3
21	MILLION LIVING WITH TYPE 2 DIABETES. THESE NUMBERS
22	ARE NOT JUST STATISTICS. THEY REPRESENT A
23	SUBSTANTIAL PORTION OF OUR COMMUNITY WHOSE QUALITY
24	OF LIFE COULD POTENTIALLY BE DRAMATICALLY IMPROVED
25	THROUGH FOCUSED EFFORTS. HOWEVER, IN ORDER TO
	10

1	UNDERSTAND WHETHER CIRM'S EFFORTS SHOULD BE
2	PRIORITIZED THERE, WE ALSO LOOKED AT OTHER FACTORS
3	THAT COMBINED CAN HELP US EVALUATE THE IMPACT AND
4	FEASIBILITY OF OUR PROPOSED RECOMMENDATIONS.
5	FOR EXAMPLE, WE LOOKED INTO STEM CELL
6	MODELING AND WHETHER EFFECTIVE STEM CELL MODELS
7	EXIST FOR EACH DISEASE, WHICH IS PIVOTAL FOR
8	ADVANCING CIRM-FUNDED RESEARCH INTO DISEASE
9	MECHANISMS IF THAT IS WHAT WE PROPOSE. SO FOR
10	CONDITIONS LIKE TYPE 1 AND 2 DIABETES OR
11	OSTEOARTHRITIS, LIVER FIBROSIS, ALZHEIMER'S
12	DISEASE-RELATED DEMENTIAS, AND CARDIOVASCULAR
13	DISEASE, WE HAVE STEM CELL MODELS THAT ARE VALIDATED
14	AND COULD BE LEVERAGED FOR DISCOVERY OF DISEASE
15	MECHANISMS, NOVEL TARGETS, BIOMARKERS, AND LEVERAGE
16	OF THE CONSORTIA EXTERNAL DATA THROUGH COLLABORATIVE
17	EFFORTS TO ACCELERATE RESEARCH IN A FOCUSED WAY.
18	FOR OTHERS WE DIDN'T HAVE THAT.
19	ANOTHER ELEMENT THAT WE SUMMARIZED IN THE
20	TABLE IS THE BIOMARKER NEEDS TO ENHANCE EARLY
21	DETECTION AS WELL AS TREATMENT EFFECTIVENESS. THIS
22	IS PARTICULARLY CRUCIAL FOR CONDITIONS LIKE ASTHMA,
23	STROKE, ALZHEIMER'S DISEASE-RELATED DEMENTIAS, LIVER
24	FIBROSIS, AND OTHERS WHERE HIGH BIOMARKER NEEDS
25	ALIGN WITH OUR OBJECTIVES TO REFINE DIAGNOSTIC AND

1	THERAPEUTIC STRATEGIES.
2	THE ECONOMIC BURDEN OF THESE DISEASES WAS
3	ALSO EVALUATED. AND THESE FIGURES NOT ONLY
4	HIGHLIGHT THE FINANCIAL IMPACTS, BUT ALSO UNDERSCORE
5	WHERE OUR RESEARCH INVESTMENTS CAN HELP REDUCE COST
6	OVER TIME. NOW, THIS IS A GLOBAL ECONOMIC BURDEN.
7	SO IN SOME CASES, LIKE HYPERTENSION, PER-PATIENT IS
8	LESS THAN THE OVERALL IT MIGHT BE LARGER THAN
9	ANOTHER LIKE MULTIPLE SCLEROSIS. SO THAT'S JUST
10	SOMETHING TO CONSIDER.
11	FINALLY, WE CONSIDERED NIH 2023 SPENDING
12	AND COMPETITIVE INDUSTRY LANDSCAPE, WHICH IS NOT
13	SHOWN HERE BECAUSE IT WAS A LOT MORE COMPLEX TO ADD.
14	THE NIH SPENDING SHOWN HERE IS FOR ALL THE
15	MODALITIES AND ALL PIPELINE DEVELOPMENT AREAS FROM
16	DISCOVERY TO CLINICAL AND INFRASTRUCTURE. THIS IS
17	TO SAY THAT WE ARE PROBABLY NOT COMPARING APPLES TO
18	APPLES, BUT IT GIVES US AN INDICATION OF ALIGNMENT
19	AT A VERY HIGH LEVEL WITH SOME OF THE GAPS AND NEEDS
20	THAT MIGHT BE THERE. NEXT SLIDE.
21	THIS IS THE SECOND SLIDE SUMMARY TABLE
22	REPRESENTING THE MOST COMMON CANCERS AFFECTING
23	CALIFORNIANS. THIS IS NOT TO DRAW ATTENTION TO
24	CANCER. WE COULD NOT JUST DRAW ALL THE DATA INTO
25	ONE SLIDE ALONE. AS A REFERENCE FOR SCALE, IF YOU

1	GO TO THE PREVIOUS SLIDE, SARA, TYPE 1 DIABETES
2	AFFECTED 290,000 PATIENTS LAST YEAR. SO NOW IF YOU
3	GO BACK TO THIS ONE, WE HAVE THE DIFFERENT CANCERS
4	THAT ARE AROUND THE LEVEL OF PATIENT COUNT LIKE TYPE
5	1 DIABETES. SO IF WE FOLLOW AT SCALE, IT COULD BE A
6	SMALLER BAR.
7	THIS TABLE SHOWS THE CANCERS AFFECTING
8	MOST CALIFORNIANS, SHOWING STEM CELL AMENABILITY.
9	ALL OF THEM ARE AMENABLE. ALL OF THEM HAVE MODELS.
10	AND BIOMARKER NEED AND SOCIOECONOMIC DISEASE BURDEN.
11	NOT SHOWN HERE IS THE CIRM CANCER PORTFOLIO WHICH IS
12	VERY LARGE. CIRM HAS INVESTED IN ABOUT 130 AWARDS
13	AND MORE THAN HALF A BILLION DOLLARS IN CANCER
14	RESEARCH WITH A BROAD RANGE OF SUBCATEGORIES. AND
15	THE LARGEST INVESTMENT IS ALSO THE LOW HANGING FRUIT
16	IN CELL AND GENE THERAPIES, WHICH IS
17	LEUKEMIA/LYMPHOMA FOLLOWED BY BRAIN CANCER.
18	THE NIH, AS A REFERENCE, SPENT IN CANCER
19	FOR THE DISEASE IS SHOWN HERE AS WELL, AND WE FOUND
20	THAT SOME OF THE CANCERS, SUCH AS MELANOMA, ARE
21	FUNDED AT THE LOWER LEVEL COMPARED WITH AND WE
22	COULDN'T FIND THE NUMBERS, BUT IT WAS A SMALL NUMBER
23	FOR MELANOMA.
24	NOW, JUST AS AN IDEA, NCI, THE NATIONAL
25	CANCER INSTITUTE, HAD AN APPROPRIATION BY 2020 FOR

1	BUDGET OF 7.2 BILLION IN BUDGET.
2	SO INTRODUCING THE NEXT SLIDE, WE ALSO
3	EVALUATED WHERE THE INDUSTRY IS CONCENTRATING ITS
4	INVESTMENTS, PARTICULARLY IN THE CONTEXT OF CELL AND
5	GENE THERAPIES. ALTHOUGH THE DETAILED LANDSCAPE OF
6	INDUSTRY INVESTMENTS IS COMPLEX AND NOT FEASIBLE TO
7	BE DISPLAYED IN JUST ONE SLIDE, WE PERFORMED AN
8	EXTENSIVE ANALYSIS THAT HAS ALLOWED US TO IDENTIFY
9	KEY GAPS AND BOTTLENECKS WHERE CIRM CAN EFFECTIVELY
10	INTERVENE TO FACILITATE THE ADVANCEMENT OF CELL AND
11	GENE THERAPIES. AND BY UNDERSTANDING THESE AREAS,
12	WE ENSURE OUR INVESTMENTS WILL NOT JUST BE FILLING
13	CURRENT NEEDS, BUT ALSO STRATEGICALLY POSITIONED TO
14	ADDRESS FUTURE CHALLENGES IN THE HEALTHCARE
15	ECOSYSTEM.
16	SO THIS SLIDE PRESENTS A SUMMARY TABLE OF
17	TECHNOLOGY GAPS IN THE FIELD OF REGENERATIVE
18	MEDICINE FOR THE MOST COMMON DISEASES AFFECTING
19	CALIFORNIANS. THAT, AGAIN, DOESN'T MEAN THAT WE
20	WANT TO FOCUS ON THOSE DISEASES. WE ARE JUST TRYING
21	TO EXTRACT WHAT ARE THE TECHNOLOGY GAPS AND
22	BOTTLENECKS THAT THESE DISEASES PRESENT THAT WE CAN
23	THEN FIGURE OUT A WAY TO HAVE A FOCUSED APPROACH IN
24	TERMS OF IN THIS CASE TECHNOLOGICAL PLATFORMS. THE
25	SELECTED CANCERS ARE ON THE BOTTOM. WE PULLED THEM
	22

1	ALTOGETHER, AND THEY INCLUDE THE ONES THAT WE
2	PREVIOUSLY SHOWED IN THE PAST SLIDE.
3	SO BY UNDERSTANDING THESE AREAS, WE ENSURE
4	THAT OUR INVESTMENTS ARE JUST NOT FILLING CURRENT
5	NEEDS, BUT WE WILL BE POSITIONED TO ADDRESS FUTURE
6	CHALLENGES. EACH CRITERION IS MARKED WITH A
7	CHECKMARK, AND IT MEANS THAT THERE IS A GAP IN THAT
8	TECHNOLOGY FOR THAT DISEASE.
9	AND IN GREEN BACKGROUND, THE TWO COLUMNS
10	ARE TECHNOLOGY GAPS THAT ARE COMMON TO MANY DISEASES
11	AFFECTING CALIFORNIANS. THESE AREAS COULD BE SOME
12	OF THE ONES THAT WE COULD PROPOSE AS SPECIFIC FOCUS,
13	SOME LIKE DELIVERY/SPECIFICITY OF METHODS AND
14	EFFECTIVENESS OF DELIVERY OF THE CELLS TO TARGET
15	AREA OR SYSTEMS IN THE BODY OR SCALABLE
16	MANUFACTURING. THE NEXT ONE, NEXT SLIDE.
17	IN THIS SLIDE WE IDENTIFY THE MAJOR
18	KNOWLEDGE GAPS THAT CURRENTLY LIMIT OUR ABILITY TO
19	EFFECTIVELY TREAT A RANGE OF DISEASES WITH
20	REGENERATIVE MEDICINE TECHNIQUES. AND FOR EACH
21	DISEASE LISTED, A CHECKMARK IDENTIFIES, AGAIN,
22	SPECIFIC AREAS WHERE THE UNDERSTANDING IS
23	INSUFFICIENT AND REPRESENTS A BOTTLENECK IN OUR
24	ABILITY TO DEVELOP EFFECTIVE THERAPIES. SO IN
25	GENERAL, WE CAN SEE THREE VERY COMMON KNOWLEDGE GAPS

1	FOR ALL THESE DISEASES THAT ARE COMMON TO
2	CALIFORNIANS.
3	DISEASE HETEROGENEITY IS ONE OF THEM,
4	BASICALLY THE VARIABILITY WITHIN THE DISEASE
5	CATEGORY THAT CAN AFFECT TREATMENT RESPONSE AND
6	EFFICACY. SECOND ONE IS DISEASE MECHANISM. AND THE
7	THIRD ONE IS IMMUNE RESPONSE.
8	SO AS A REMINDER, THIS WAS A SNAPSHOT OF A
9	LOT OF DATA LEADING TO RECOMMENDATIONS THAT WILL BE
10	INTRODUCED IN THE NEXT FEW SLIDES.
11	SO LET'S GO NOW INTO THE RECOMMENDATIONS.
12	HOW ARE WE WITH TIME? IT'S 9:30. I SHOULD BE
13	FINISHED IN THE NEXT 15 MINUTES. IF I CAN, I'LL GO
14	VERY FAST.
15	SO WE'VE ALL SEEN THIS GOAL. LET'S GO TO
16	THE NEXT SLIDE. BASED ON THE DATA WITH REGARDS TO
17	THE FIRST GOAL, OUR FIRST RECOMMENDATION IS TO
18	INCREASE RESEARCH TO UNCOVER CROSS-DISEASE
19	MECHANISMS AND INTERACTIONS, AIMING FOR
20	BREAKTHROUGHS IN IDENTIFYING NEW DISEASE MECHANISMS,
21	TARGETS, AND BIOMARKERS, LEVERAGING DATA ACROSS
22	DISEASES AND WITH OTHER CONSORTIA WHICH THIS COULD
23	BE APPLICABLE TO BOTH PREVALENT AND RARE DISEASES.
24	THE OBJECTIVE HERE COULD BE TO ENHANCE
25	RESEARCH TO EXPLORE CROSS-DISEASE MECHANISMS,

1	SYSTEMS AND INTERACTIONS, AIMING FOR BREAKTHROUGHS
2	IN NEW DISEASE MECHANISMS, TARGETS, AND BIOMARKERS.
3	AND WE WOULD UTILIZE CROSS-DISEASE DATA AND
4	COLLABORATE WITH VARIOUS CROSS-FUNDED RESEARCHERS
5	IN CALIFORNIA BY CIRM AND OTHERS, ALSO WITH OTHER
6	CONSORTIA AND PROJECTS TO MAXIMIZE OUR RESEARCH
7	OUTCOMES. CLICK NEXT.
8	SO THE RECOMMENDATION IS TO SUPPORT
9	COMPREHENSIVE DISCOVERY RESEARCH THROUGH STRUCTURED
10	INITIATIVES SUCH AS THE ONE THAT WE DEVELOPED OVER
11	THE PAST YEAR THAT WE HAVE PILOTED WITH THE REMIND
12	PROGRAM, BUT THAT WE COULD EXTEND TO OUR DISCOVERY
13	RESEARCH. AND THE APPROACH COULD BE TO ENCOURAGE
14	COLLABORATIVE, MULTIDISCIPLINARY INNOVATION,
15	LEVERAGING STEM CELL AND GENETIC RESEARCH ACROSS
16	DIVERSE DISCIPLINES AND DISEASE INDICATIONS. NEXT
17	SLIDE.
18	IN ORDER TO ACHIEVE THE CROSS-DISEASE DATA
19	AND COLLABORATION YOU CAN CLICK THE NEXT. THANK
20	YOU, SARA WE ARE PROPOSING A SECOND
21	RECOMMENDATION THAT COULD MATERIALIZE IN THE FORM OF
22	ESTABLISHING A DATA COORDINATING AND MANAGEMENT
23	CENTER, A DCMC. THE OBJECTIVE HERE COULD BE TO
24	STREAMLINE THE DATA MANAGEMENT TO ENHANCE EACH
25	UTILITY ACROSS DISEASE DATA AND ALSO TO LEVERAGE

1	OTHER DATA. AND THE APPROACH COULD BE TO FUND AND
2	DEVELOP A CENTRAL HUB FOR DATA COORDINATION,
3	FACILITATING BETTER INTEGRATION WITH CONSORTIA AND
4	WITHIN CIRM-FUNDED RESEARCH.
5	THIS SECOND RECOMMENDATION HAS AN EMPHASIS
6	ON VALIDATION AND REPRODUCIBILITY OF RESEARCH
7	RESULTS, WHICH IS, WE THINK, INDEED A PIVOTAL NEED
8	TO MOVE RESEARCH THAT WILL ULTIMATELY LEAD TO
9	SUCCESSFUL THERAPIES. AND THIS WAS A RECOMMENDATION
10	FROM OUR NEW CO-CHAIRS OF THE NEURO TASK FORCE, DR.
11	PAT LEVITT AND DR. CAROLYN MELTZER, WHO WHEN WE WERE
12	DISCUSSING SOME OF THIS, THEY BROUGHT THIS UP. AND
13	WE WOULD LIKE TO THANK THEM. ALSO, WE WOULD LIKE TO
14	WELCOME THEM AS NEW CO-CHAIRS.
15	NEXT SLIDE IS THE SECOND GOAL. AND WE CAN
16	GO NOW INTO THE NEXT SLIDE, AND YOU CAN CLICK IT AS
17	WELL SO THAT WE CAN JUST PRESENT. SO BROAD
18	APPLICABILITY OF CELL AND GENE THERAPIES FOR RARE
19	AND PREVALENT DISEASES WILL REQUIRE, AS WE ALREADY
20	SAID, IMPLEMENTATION OF NEW TECHNOLOGIES AND
21	TECHNOLOGY PLATFORMS THAT CAN ENSURE THE SAFETY,
22	EFFICACY, AND RELIABILITY OF MULTIPLE CELL AND GENE
23	THERAPIES.
24	CURRENTLY OUR CIRM PROGRAMS FOCUS THROUGH
25	DIFFERENT PROGRAMS ON SUPPORTING TECHNOLOGIES IN THE

1	CONTEXT OF SPECIFIC THERAPEUTIC AREA CANDIDATE
2	PROJECTS OR ALSO OFFERS LIMITED FUNDING FOR EARLY
3	STAGE DISCOVERY AND TOOL DEVELOPMENT. AND THIS
4	CURRENT APPROACH HAS NOT EFFECTIVELY ENCOURAGED
5	MULTIPLE STAKEHOLDER COLLABORATIONS BETWEEN ACADEMIA
6	AND INDUSTRY OR OTHERS, WHICH WE THINK, FROM WHAT
7	WE'VE SEEN, IS CRUCIAL FOR THE TRANSLATABILITY AND
8	DEVELOPMENT AND SUCCESS OF THESE TECHNOLOGIES.
9	THE PROPOSED RECOMMENDATION HERE COULD AIM
10	TO REFINE CIRM'S STRATEGIC APPROACH BY ADDRESSING
11	SPECIFIC LIMITATIONS THAT WE HAVE IDENTIFIED. SO
12	THE PROPOSAL WOULD BE TO INVEST IN MULTIDISCIPLINARY
13	TECHNOLOGY PLATFORM-FOCUSED INITIATIVES WITH THE
14	OBJECTIVE TO EXPEDITE THE DEVELOPMENT AND
15	APPLICATION OF THESE TECHNOLOGIES THAT ENHANCE THE
16	SAFETY, EFFICACY, OR QUALITY OF CELL AND GENE
17	THERAPIES. AND THE APPROACH COULD BE TO ENCOURAGE
18	MULTIDISCIPLINARY, MULTISTAKEHOLDER
19	ACADEMIA/INDUSTRY COLLABORATIONS TO DEVELOP PLATFORM
20	TECHNOLOGIES THAT BROADLY IMPACT PRECLINICAL AND
21	DEVELOPMENT OF MULTIPLE THERAPIES FOR MULTIPLE
22	DISEASES.
23	THIS COULD REQUIRE A PILOT INITIATIVE, AN
24	INFRASTRUCTURE TECHNOLOGY PLATFORM PROGRAM THAT
25	COULD BRIDGE THE GAP BETWEEN RESEARCH AND

1	COMMERCIALIZATION BY FOSTERING PARTNERSHIPS BETWEEN
2	THESE ACADEMIC RESEARCHERS AND INDUSTRY RESEARCHERS
3	AND PROFESSIONALS. AND THE APPROACH COULD BE TO
4	SUPPORT MULTIPLE STAKEHOLDER TECHNOLOGY INCUBATION
5	PROGRAMS TO ACHIEVE, DEFINE TECHNOLOGY READINESS
6	LEVELS, THEREBY FACILITATING RAPID APPLICATION IN
7	CELL AND GENE THERAPY DEVELOPMENT.
8	THIS COULD BE A NEW PROGRAM. THIS IS NOT
9	EVEN A CONCEPT YET. THIS IS THE IDEA. THE
10	RECOMMENDATION FOR DISCUSSING AND PRESSURE TESTING,
11	I JUST WANT TO MAKE THIS CLEAR BECAUSE WE HAVE SOME
12	IDEAS, BUT IT'S NOT FULLY COOKED BY ANY MEANS. NEXT
13	SLIDE.
14	THIS IS ONLY TO SHOW THE PROPOSED CHANGES
14 15	THIS IS ONLY TO SHOW THE PROPOSED CHANGES FROM WHAT WE HAVE RIGHT NOW FOR GOAL 1 TO WHAT WE
15	FROM WHAT WE HAVE RIGHT NOW FOR GOAL 1 TO WHAT WE
15 16	FROM WHAT WE HAVE RIGHT NOW FOR GOAL 1 TO WHAT WE COULD BE PROPOSING AND THAT COULD MEAN. SO RIGHT
15 16 17	FROM WHAT WE HAVE RIGHT NOW FOR GOAL 1 TO WHAT WE COULD BE PROPOSING AND THAT COULD MEAN. SO RIGHT NOW DOES NOT MEAN THAT WE'RE NOT DOING IT WELL, BUT
15 16 17 18	FROM WHAT WE HAVE RIGHT NOW FOR GOAL 1 TO WHAT WE  COULD BE PROPOSING AND THAT COULD MEAN. SO RIGHT  NOW DOES NOT MEAN THAT WE'RE NOT DOING IT WELL, BUT  WE CAN DO IT BETTER. RIGHT? SO WE DO A LOT OF
15 16 17 18 19	FROM WHAT WE HAVE RIGHT NOW FOR GOAL 1 TO WHAT WE COULD BE PROPOSING AND THAT COULD MEAN. SO RIGHT NOW DOES NOT MEAN THAT WE'RE NOT DOING IT WELL, BUT WE CAN DO IT BETTER. RIGHT? SO WE DO A LOT OF FOUNDATIONAL RESEARCH THROUGH THE DISC-0. IT DOES
15 16 17 18 19 20	FROM WHAT WE HAVE RIGHT NOW FOR GOAL 1 TO WHAT WE COULD BE PROPOSING AND THAT COULD MEAN. SO RIGHT NOW DOES NOT MEAN THAT WE'RE NOT DOING IT WELL, BUT WE CAN DO IT BETTER. RIGHT? SO WE DO A LOT OF FOUNDATIONAL RESEARCH THROUGH THE DISC-O. IT DOES NOT HAVE DISEASE MECHANISTIC FOCUS. IT'S FOCUSED ON
15 16 17 18 19 20 21	FROM WHAT WE HAVE RIGHT NOW FOR GOAL 1 TO WHAT WE COULD BE PROPOSING AND THAT COULD MEAN. SO RIGHT NOW DOES NOT MEAN THAT WE'RE NOT DOING IT WELL, BUT WE CAN DO IT BETTER. RIGHT? SO WE DO A LOT OF FOUNDATIONAL RESEARCH THROUGH THE DISC-O. IT DOES NOT HAVE DISEASE MECHANISTIC FOCUS. IT'S FOCUSED ON SMALL COLLABORATIONS OF ONE OR TWO INVESTIGATORS.
15 16 17 18 19 20 21	FROM WHAT WE HAVE RIGHT NOW FOR GOAL 1 TO WHAT WE COULD BE PROPOSING AND THAT COULD MEAN. SO RIGHT NOW DOES NOT MEAN THAT WE'RE NOT DOING IT WELL, BUT WE CAN DO IT BETTER. RIGHT? SO WE DO A LOT OF FOUNDATIONAL RESEARCH THROUGH THE DISC-O. IT DOES NOT HAVE DISEASE MECHANISTIC FOCUS. IT'S FOCUSED ON SMALL COLLABORATIONS OF ONE OR TWO INVESTIGATORS. THERE'S NO MULTIDISCIPLINARITY. WE DO NOT LEVERAGE
15 16 17 18 19 20 21 22 23	FROM WHAT WE HAVE RIGHT NOW FOR GOAL 1 TO WHAT WE COULD BE PROPOSING AND THAT COULD MEAN. SO RIGHT NOW DOES NOT MEAN THAT WE'RE NOT DOING IT WELL, BUT WE CAN DO IT BETTER. RIGHT? SO WE DO A LOT OF FOUNDATIONAL RESEARCH THROUGH THE DISC-O. IT DOES NOT HAVE DISEASE MECHANISTIC FOCUS. IT'S FOCUSED ON SMALL COLLABORATIONS OF ONE OR TWO INVESTIGATORS. THERE'S NO MULTIDISCIPLINARITY. WE DO NOT LEVERAGE EXTERNAL OR EVEN RESOURCES AMONGST US. WE DON'T

1	THROUGH DISC4, DISC5, SO EXTENDING THE WHOLE NEW
2	PILOT CONCEPT, PROGRAM THAT WE HAVE STARTED TO
3	INCLUDE NOW ALL DISEASE AREAS COULD ALLOW US TO DO
4	THAT THROUGH LARGE COLLABORATIVE PROJECTS FOCUSED ON
5	DISEASE MECHANISMS, LEVERAGING RESOURCES BETWEEN THE
6	DIFFERENT DISEASES THAT CIRM FUNDS, AND ALSO
7	FOCUSING NOW THAT IF WE HAVE POWER WITH THE DATA, WE
8	CAN THEN TRY TO FIGURE OUT WHAT ARE PARTNERSHIPS AND
9	EXTERNAL RESOURCES WE CAN WORK WITH.
10	AND DISC5 COULD BE MORE SMALL. NOT
11	EVERYBODY HAS A LARGE COLLABORATIVE PROJECT. SOME
12	PEOPLE NEED FUNDING FOR EXPLORATORY PROJECTS, MORE
13	RISKY, BUT POTENTIALLY MORE REWARDING, FOCUSED ON
14	DISEASE MECHANISMS.
15	NOW, TO DO ALL THAT, WE ALSO NEED DATA
16	SHARING, BUT ALSO COORDINATION AND MANAGEMENT OF
17	THAT DATA. AND THE DCMC IS NOT AN ISOLATED THING.
18	IT'S BASICALLY THE NEXT STEP IN A DATA
19	INFRASTRUCTURE PROGRAM THAT WE HAVE BEEN PUTTING
20	TOGETHER SINCE WE STARTED THE SECOND PHASE OF CIRM,
21	THE CIRM 3.0. SO WE STARTED WITH DATA SHARING AND
22	MANAGEMENT PLANS. SO RIGHT NOW, SAME AS WITH NIH
23	AND ALIGNED WITH NIH, WE HAVE REQUIREMENTS TO DETAIL
24	DATA SHARING PLANS AND ALL THE METADATA. WE HAVE
25	NOW, AS YOU VOTED THROUGH THE GOVERNANCE

1	SUBCOMMITTEE, A CONTRACT TO EXTEND WITH RANCHO
2	BIOSCIENCES. THEY ARE DEVELOPING THIS DATA
3	DASHBOARD. AND THE NEXT PHASE COULD BE HAVING A
4	DATA COORDINATION AND MANAGEMENT CENTER WITH A
5	KNOWLEDGE PLATFORM THAT COULD ENABLE AND ENCOURAGE
6	DATA REUSE AND INTEGRATION WITH EXTERNAL RESOURCES.
7	THE NEXT SLIDE SHOWS HOW WE COULD
8	TRANSITION FOR THE SECOND GOAL RECOMMENDATIONS FROM
9	THE BROAD APPROACH THAT WE HAVE NOW WHERE CERTAIN
10	TECHNOLOGY ASPECTS WERE ADDRESSED THROUGH DIFFERENT
11	PROGRAMS, DISC2, TRAN1, 2, 3, 4, CLIN1 WITHOUT
12	DISCONTINUING FOCUS. WE COULD GO NOW TO A MORE
13	TARGETED STRATEGY. AND THIS NEW DIRECTION LEVERAGES
14	MULTIDISCIPLINARY COLLABORATIONS AND INDUSTRY
15	PARTNERSHIPS TO ENHANCE SPECIFICITY AND
16	EFFECTIVENESS IN OUR PROJECTS, ACCELERATE THE
17	DEVELOPMENT AND VALIDATION OF TECHNOLOGIES THAT WILL
18	SPECIFICALLY ADVANCE SAFETY, EFFICACY, AND QUALITY
19	OF CELL AND GENE THERAPIES. NEXT SLIDE.
20	NOW WE ARE GETTING I THINK WE WILL MAKE
21	IT IN HALF OF THE MEETING. SO WE DID WELL.
22	DISCUSSION AND NEXT STEPS. THE TIMELINE
23	THAT WE HAVE, THIS SLIDE SHOWS THAT TODAY WE WILL BE
24	REVIEWING AND DISCUSSING THESE POTENTIAL
25	RECOMMENDATIONS AND ANSWERING QUESTIONS. THE NEXT

1	TIME THAT WE WOULD HEAR ABOUT THESE GOALS 1 AND 2
2	COULD BE AT THE AUGUST NEURO TASK FORCE/SCIENCE
3	SUBCOMMITTEE JOINT MEETING WHERE WE WILL BE
4	INTRODUCING GOALS 3 AND 4. THAT'S GOING TO BE A
5	VERY LONG MEETING. IT NEEDS TO BE BECAUSE IT'S A
6	VERY PACKED MEETING. BUT WE WILL ALSO BE PRESENTING
7	ANY UPDATES ON WHAT WE HAVE DISCUSSED TODAY.
8	SO I DON'T WANT TO TAKE MORE TIME. WE
9	WILL SEND THESE SLIDES. SO THE NEXT SLIDE IS JUST
10	SHOWING THE TIMELINE AND WHERE THINGS ARE. ON
11	AUGUST 7TH WE WILL HAVE A MEETING WITH THE
12	ACCESSIBILITY AND AFFORDABILITY WORKING GROUP TO GO
13	OVER GOAL NO. 5. AND THEN AT THE 16TH MEETING, WE
14	WILL HAVE GOALS 3 AND 4 WHICH ARE THROUGH CELL AND
15	GENE THERAPY FOCUSED GOALS. AND I THINK THAT'S IT
16	FROM OUR END IN TERMS OF THE PRESENTATION. THANK
17	YOU VERY MUCH FOR LISTENING AND APPRECIATE IT.
18	CHAIRMAN FISCHER-COLBRIE: GREAT. THANK
19	YOU, ROSA, FOR AN EXCELLENT PRESENTATION. AND I
20	WOULD LIKE TO CONSIDER AND DISCUSS HERE, BUT LET'S
21	OPEN IT UP FOR QUESTIONS AND COMMENTS BY THE GROUP.
22	I SEE PAT LEVITT.
23	DR. LEVITT: YEAH. SO, ROSA, I MEAN I'VE
24	HEARD THIS BEFORE, AND IT'S BREATHTAKING EACH TIME I
25	HEAR IT. THERE'S A TON OF WORK THAT WAS DONE BY THE

1	TEAM. CONGRATULATIONS ON PUTTING THIS SLIDE UP THAT
2	LISTS ALL OF THE AMAZING TEAM MEMBERS THAT WORKED IN
3	A REALLY INTEGRATED WAY TO GENERATE THIS. AND FOR
4	THOSE OF US WHO DO DATA ANALYSES WOULD KNOW THAT
5	THAT WAS AN INCREDIBLY HEAVY LIFT.
6	SO I HAVE TWO THINGS TO BRING UP. THE
7	FIRST IS IN THE ACADEMIA/INDUSTRY PARTNERSHIPS, ONE
8	OF THE THINGS THAT WE'VE TALKED ABOUT
9	PREVIOUSLY MARK HAS A FONDNESS FOR WHEN I MENTION
10	THIS ONE OF THE MAJOR BOTTLENECKS IS
11	REPRODUCIBILITY FROM EXPERIMENT STUDIES THAT REPORT
12	FROM ACADEMIA THAT THEN GET TRANSFERRED AND
13	TRANSLATED TO INDUSTRY THAT CAN'T REPRODUCE THE
14	DATA. THEY CAN'T REPRODUCE THE EXPERIMENTS. IT
15	HAPPENS FAR MORE ON OFTEN THAN THEY CAN REPRODUCE,
16	AND THAT'S A HUGE BOTTLENECK. IT'S BEEN WRITTEN
17	ABOUT. I THINK, MARK, YOU'VE WRITTEN ABOUT IT. AND
18	IT'S A HUGE BOTTLENECK.
19	SO I'M WONDERING WHETHER THE
20	ACADEMIA/INDUSTRY PARTNERSHIPS, WHICH ARE NOT
21	MENTIONED IN THE DISC4 AND DISC5, MIGHT BE
22	CONSIDERED THERE AS A WAY OF GETTING INDUSTRY TO
23	WORK WITH ACADEMIA ON BOARD FOR THESE VERY TARGETED,
24	FOCUSED DISC GRANTS ON DISEASE MECHANISMS SO THAT WE
25	SKIP THE STEP IN WHICH INDUSTRY THEN SPENDS TWO

1	YEARS TRYING TO REPRODUCE SOMETHING THAT THEY CAN'T
2	REPRODUCE AND THEN THEY GIVE UP. UNDERSTANDABLY,
3	THEY GIVE UP. SO THAT'S ONE COMMENT TO CONSIDER,
4	THAT THOSE PARTNERSHIPS MAY BE REALLY IMPORTANT FOR
5	ADDRESSING THIS REPRODUCIBILITY BOTTLENECK. AND
6	OTHERS MAY FEEL THE SAME WAY.
7	THE OTHER IS THE EFFORT FOR NEW
8	STREAMLINING DATA MANAGEMENT WHERE YOU HAVE THIS
9	INTEGRATED BETWEEN THOSE WHO ARE CIRM-FUNDED AND THE
10	EXTERNAL SOURCES. I ASSUME EARLY ON THERE'S GOING
11	TO BE WRITTEN THERE WILL BE MOU'S TO MAKE SURE
12	THAT WE GET THE EXTERNAL AGREEMENTS THAT THEY'RE
13	GOING TO PARTICIPATE IN THIS.
14	BUT ONE THING I WAS THINKING ABOUT FOR THE
15	FIRST TIME, BECAUSE I'VE HEARD THIS BEFORE AND
16	HADN'T THOUGHT ABOUT IT, IS WHETHER WE WOULD
17	CONSIDER DATA SCIENCE GRANTS THAT WOULD BE LINKED TO
18	UTILIZING THAT PLATFORM WITH ENORMOUS AMOUNTS OF
19	DATA RATHER THAN LEAVING IT TO THE SCIENTISTS TO
20	THEN GO IN AND DO THE OR FIGURE OUT HOW THEY'RE
21	GOING TO COLLABORATE WITH THE DATA SCIENTISTS. WHY
22	NOT HAVE DATA SCIENCE-FOCUSED GRANTS THAT WOULD
23	UTILIZE WHERE THE CHALLENGE WOULD BE TO UTILIZE
24	THAT PLATFORM TO THEN REALLY ANALYZE THE DATA IN
25	WAYS THAT WE ARE NOT DOING NOW TO REALLY TAKE

1	ADVANTAGE OF THAT.
2	AND SO THAT WOULD NOT MAKE A HUGE DENT IN
3	THE PORTFOLIO, BUT WOULD BRING DATA SCIENTISTS INTO
4	THE FOLD TO GREATER LEVERAGE ON WHAT WE'RE TRYING TO
5	DO. I THINK IT'S A GREAT EFFORT TO INTEGRATE IN
6	THIS WAY. YOU'VE BEEN TALKING ABOUT IT FOR WE'VE
7	BEEN TALKING ABOUT IT FOR SEVERAL YEARS AT LEAST
8	SINCE I'VE BEEN ON THE BOARD. SO MAYBE THINK ABOUT
9	THAT AS ANOTHER POSSIBLE MECHANISM FOR FUNDING.
10	I'LL STOP THERE.
11	DR. CANET-AVILES: FANTASTIC POINTS AS
12	ALWAYS, PAT. CAROLYN, YOU HAVE YOUR HAND RAISED.
13	DO YOU MIND IF I ANSWER THESE?
14	SO I THINK THE REPRODUCIBILITY BOTTLENECK,
15	AND I COULD SEE MY COLLEAGUE SHYAM, HE WAS DOING
16	LIKE THIS WITH HIS HEAD, ON THE INDUSTRY AND
17	ACADEMIA. I ACTUALLY AND I'M IMAGINING YOU'RE ALSO
18	AGREEING, SHYAM, I THINK THAT THIS IS A VERY GOOD
19	IDEA THAT YOU ARE PROPOSING BECAUSE AND IT
20	REMINDED ME OF THE AMP PARTNERSHIPS, THE ACCELERATED
21	MEDICINE PARTNERSHIPS, AT THE NIH THAT WE WERE
22	DEVELOPING IN DIFFERENT DISEASES. SO THAT IS
23	SOMETHING AND IN OUR CASE WE COULD HAVE THE
24	FREEDOM TO BRING IN INDUSTRY, ANYBODY THAT WANTS TO
25	COME BECAUSE WE ARE OFFERING FUNDING. IT'S NOT LIKE

1	WE ARE GATHERING PEOPLE TOGETHER AND ASKING THEM FOR
2	THE FUNDING.
3	SO I THINK THAT'S A GOOD IDEA, AND IT
4	COULD BE A REQUIREMENT THAT WE COULD ADD IN THE
5	PROGRAM ELIGIBILITY, THAT THERE HAS TO BE A
6	MULTIDISCIPLINARY HAS TO COME ALSO, THE INDUSTRY
7	PARTNER, THAT WILL PROVIDE FROM ONE SIDE WE WILL
8	HAVE THE ACADEMIC, BUT THEN THE INDUSTRY PARTNER IS
9	GOING TO COME WITH FOCUS ON REFINING AND HOW THIS
10	COULD MOVE THE NEEDLE AND ALSO MAKING SURE THAT WE
11	CAN MAKE IT REPRODUCIBLE. SO YEAH, DEFINITELY.
12	DR. LEVITT: AND THEY CAN BE INVOLVED IN
13	STUDY DESIGN ISSUES THAT
14	DR. CANET-AVILES: YEAH.
15	DR. LEVITT: THAT SEEM TO BE A MAJOR
16	CHALLENGE.
17	DR. CANET-AVILES: YES.
18	DR. LEVITT: WHY REPRODUCIBILITY IS SO
19	DIFFICULT. BECAUSE THE STUDY DESIGNS WORK WELL IN
20	ACADEMIA, BUT NOT NECESSARILY WORK WHEN YOU TRY TO
21	SCALE. SO THAT'S ONE THING, YEAH.
22	DR. CANET-AVILES: AND THE FEASIBILITY
23	PROBABLY TO KNOW WHETHER THE RESULT WILL BE
24	APPLICABLE IF THEY HAVE THIS DISEASE AREA IN THEIR
25	FOCUS.

1	IN TERMS OF EXTERNAL DATA, THE DATA
2	SCIENCE GRANTS TO UTILIZE DATA, I THINK
3	THAT'S THAT COULD BE PART OF WHAT I HADN'T
4	THOUGHT ABOUT IT, BUT THIS IS SOMETHING THAT COULD
5	COME, IF WE THINK IN A MODULAR WAY, WE FIRST HAVE
6	THE DATA COORDINATING MANAGEMENT CENTER, AND THEN WE
7	HAVE A SPECIFIC COLLABORATIVE SCIENCE GRANT. BUT
8	FOR THAT THE BOTTLENECK IS THE DATA AND THE POWER OF
9	THE DATA. IF WE LOOK AT, SAY, PARKINSON'S, WE DON'T
10	HAVE ENOUGH DATA GENERATED AT CIRM. RIGHT? WE ARE
11	GOING TO HAVE THE FIRST POWERED DATA HOPEFULLY WITH
12	THE REMIND PROGRAM BECAUSE IN AUGUST WE'LL HAVE THE
13	ARS, AND YOU WILL SEE THAT MOSTLY WE GOT TWO MAIN
14	DISEASES. SO WE MIGHT BE ABLE TO GENERATE ENOUGH
15	DATA THERE THAT WE CAN GO AND COLLABORATE AND HAVE
16	THIS APPROACH.
17	SO I'LL TAKE THAT INTO THE NOTES OF WHEN
18	WE PROPOSE IT, IF THE BOARD AGREES. WHEN WE PROPOSE
19	IT, WE SHOW A PHASED APPROACH FOR THIS DATA
20	COORDINATING MANAGEMENT CENTER WITH DATA SCIENCE
21	GRANTS TO UTILIZE THE DATA.
22	AND IN TERMS OF EXTERNAL DATA, DEFINITELY
23	WE WOULD NEED MOU'S WITH DIFFERENT PARTNERS. WE ARE
24	ALREADY TALKING WITH DIFFERENT RELEVANT NIH
25	INSTITUTES. AND WE WOULD HAVE TO TALK TO OTHERS AS

1	WELL, NOT ONLY LIKE MICHAEL J. FOX OR ALZHEIMER'S
2	DISEASE ASSOCIATION OR OTHERS, RIGHT, BECAUSE
3	THERE'S ALSO THE ADDI, THE ALZHEIMER'S DISEASE
4	DISCOVERY INITIATIVE, OR THE ALS. RIGHT? SO THERE
5	IS DIFFERENT PLACES WHERE WE COULD GO, BUT WE WOULD
6	HAVE TO FIGURE OUT WHAT DO WE HAVE IN TERMS OF POWER
7	IN DATA AND WHAT ARE THE BOTTLENECKS, WHERE DO WE
8	WANT TO GO. WE WILL HAVE TO CHOOSE. BUT THAT COULD
9	BE PART OF THE PROPOSAL IN THE CONCEPT. SO THANK
10	YOU. THAT'S REALLY HELPFUL, RELEVANT.
11	DR. MELTZER: ROSA, THANK YOU SO MUCH.
12	JUST TO ECHO PAT'S COMMENTS, THE TEAM HAS DONE AN
13	INCREDIBLE JOB WITH SYNTHESIZING DATA. I DO THINK
14	IT'S WORTH SPENDING MORE TIME AT SOME POINT ON SLIDE
15	20 WHERE YOU LOOK AT THE DISEASE PATIENT IMPACT,
16	BIOMARKER NEED, ECONOMIC BURDEN AS WE CONSIDER AND
17	WHERE BIOMARKERS MAY BE AVAILABLE. ALSO MAYBE
18	THINKING ABOUT FUTURE TRENDS OF DISEASE, AGING
19	POPULATION, ALZHEIMER'S, HEALTH DISPARITIES, AND
20	INCREASING CLIMATE CHANGE. THE THINGS LIKE ASTHMA
21	BECOMING MORE PREVALENT. SO THERE'S PROBABLY SOME
22	WAYS TO PROJECT INCREASING OR DECREASING IMPACT OF
23	SOME OF THE DISEASES AND THEN ADDED WITH THE
24	TECHNOLOGY GAPS.
25	I ALSO REALLY LOVE THE IDEA OF HAVING MORE

1	MULTIDISCIPLINARY ACADEMIC/INDUSTRY PARTNERSHIPS.
2	REPRODUCIBILITY IS A HUGE ISSUE, BUT ALSO THERE ARE
3	MULTIPLE OTHER WAYS THAT INTEGRATED PARTNERSHIPS CAN
4	STREAMLINE TRANSLATION, STUDY DESIGN, POWER,
5	POPULATION, HOW THINGS ARE PLANNED FROM THE
6	BEGINNING, AND THE ACADEMIC EXPERIMENTS THAT ARE AT
7	THAT PHASE. SO SO MUCH GREAT WORK TO DO. THANK
8	YOU.
9	DR. CANET-AVILES: THANK YOU. THOSE ARE
10	FANTASTIC COMMENTS. THANK YOU, CAROLYN.
11	SHLOMO. YOU'RE MUTED.
12	DR. MELMED: THANK YOU. AND ONCE AGAIN,
13	KUDOS TO YOU AND THE TEAM. SUPER, SUPER
14	PRESENTATION. REALLY CONGRATULATIONS. WE CAN ALL
15	BE PROUD OF YOUR WORK.
16	I WANT TO COME BACK TO A COMMENT WHICH PAT
17	MADE, I THINK, LAST WEEK OR TWO WEEKS AGO IN A
18	MEETING. AND THAT IS I THINK WE HAVE TO BE
19	SUFFICIENTLY FLEXIBLE IN OUR THINKING AS TO WHAT MAY
20	HAPPEN WITH NIH. AND IF THE CURRENT NIH PROPOSAL
21	DOES GO THROUGH AS PROPOSED, WE'RE GOING TO FACE A
22	TECTONIC CHANGE IN OUR ACADEMIC MEDICINE AND
23	RESEARCHERS SUPPORTED. AND I WOULD WONDER IF THERE
24	SHOULD BE SOME ROOM OR AT LEAST A STATEMENT IN OUR
25	STRATEGIC VISION THAT WE HAVE THE SUFFICIENT

1	FLEXIBILITY TO RESPOND IN CALIFORNIA TO WHAT MAY
2	HAPPEN NATIONALLY. BECAUSE AS PAT CORRECTLY POINTED
3	OUT, THE CHANGES THAT ARE BEING PROPOSED AT NIH ARE
4	GOING TO HAVE A MAJOR, MAJOR DETRIMENTAL IMPACT ON
5	ALL MEDICAL RESEARCH AS WE KNOW IT. AND OUR PLAN AS
6	YOU PRESENTED MAY BE DEFUNCT. AND WE MAY HAVE TO
7	START FROM SCRATCH AGAIN, CREATING OUR OWN MODELS
8	TO FILL THAT VACUUM. IT MAY NOT HAPPEN. THIS IS
9	ALL FUTURISTIC, BUT THE PLANS ON THE TABLE ARE
10	PRETTY SCARY. AND I WOULD ASK US AT LEAST TO HAVE
11	SOME FLEXIBILITY IN OUR LANGUAGE THAT WE DO HAVE THE
12	ABILITY TO PIVOT IF, IN FACT, OUR SOCIETY IN
13	CALIFORNIA DEMANDS IT OF US BECAUSE WE'LL BE THE
14	ONLY ONES HERE TO CARRY THAT BURDEN.
15	PAT, I'D REALLY LIKE TO HEAR YOU EXTEND
16	YOUR THOUGHTS WHICH YOU PRESENTED OR AT LEAST RAISED
17	A COUPLE OF WEEKS AGO.
18	DR. LEVITT: I DON'T KNOW WHAT TO ADD TO
19	THAT EXCEPT THAT IT'S GOOD THAT YOU REMEMBER WHAT I
20	SAID TWO WEEKS AGO BECAUSE I CAN'T.
21	I MEAN WE HAVE SEVERAL CHALLENGES. ONE IS
22	HAVING RAPID FLEXIBILITY FOR AN ORGANIZATION LIKE
23	THIS IS ALWAYS A CHALLENGE, BUT IT HAS TO BE DONE
24	WITHIN A CONSTRAINED FRAMEWORK, RIGHT, OR THE CHARGE
25	THROUGH THE PROPOSITION IS TO FOCUS ON SPECIFIC

1	KINDS OF BIOMEDICAL RESEARCH. AND SO I THINK WE CAN
2	ADAPT TO WHATEVER DISASTERS ARE COMING OUR WAY IF
3	THAT IS PASSED.
4	I'VE HEARD FROM OUR, AND I'M SURE YOU HAVE
5	AS WELL, ALL OF US WHO HAVE RELATIONS WITH OUR
6	LEGISLATIVE FOLKS THAT THEY BELIEVE IT'S A
7	NONSTARTER BASED ON THE CURRENT CENSUS OF CONGRESS.
8	BUT THERE ARE THINGS IN THERE THAT ARE SUBTLY
9	MENTIONED, A FEW SENTENCES HERE OR THERE THAT ARE
10	REALLY DRACONIAN, A MAJOR PROBLEM. SO I WOULD SAY
11	WE CAN WE CAN HAVE LANGUAGE THAT WOULD ALLOW US
12	TO PIVOT RELATIVELY RAPIDLY AS AN ORGANIZATION,
13	KEEPING IN MIND THAT WE ARE ONLY GOING TO BE ABLE TO
14	DO THAT IN THE CONTEXT OF STEM CELL REGENERATIVE
15	MEDICINE AND GENETIC AND GENE THERAPIES.
16	BUT THE POINT YOU MADE, SHLOMO, IS REALLY
17	IMPORTANT BECAUSE PART OF WHAT WE SHOULD BE
18	PROPOSING IS MOST ALL OF OUR GRANTEES ARE ACADEMIC.
19	RIGHT. AND SO HOW ARE WE GOING TO FILL WHATEVER
20	GAPS MAY OCCUR? THAT'S PROBABLY FOR ANOTHER
21	CONVERSATION IF IT HAPPENS. BUT FLEXIBLE LANGUAGE
22	WOULD BE GOOD, AND I THINK THAT COULD BE DONE EVEN
23	WITHIN THE CONTEXT OF THE VERY SPECIFIC MODELS THAT
24	THE TEAM HAS PUT FORTH.
25	I DON'T THINK ANY OF US HAVE A CRYSTAL

1	BALL OF WHAT'S GOING TO HAPPEN. AND WE'LL KNOW
2	AFTER NOVEMBER 4. THAT'S FOR SURE. THAT'S ALL I
3	HAVE. I DON'T KNOW WHAT ELSE TO SAY EXCEPT THAT
4	IT'S NOT A HAPPY TIME IN ACADEMIA AFTER YOU READ
5	THAT.
6	DR. CANET-AVILES: YEAH. AND I THINK WE
7	WOULD, AS ALWAYS, BE VERY FLEXIBLE WITH THE LANGUAGE
8	THAT WE HAVE. RIGHT NOW WE ARE PROPOSING AN
9	APPROACH, AND THEN WE CAN PUT THE SPECIFICS WHEN WE
10	RELEASE THE DIFFERENT PROGRAM ANNOUNCEMENTS, AND
11	THERE WILL BE ENOUGH FLEXIBILITY. BUT THAT'S A
12	GREAT POINT. THANK YOU BOTH.
13	I THINK IT'S I'M NOT THE CHAIR OF
14	THE I'M SORRY, MARK. I FORGOT. AFTER I PRESENT,
15	I GET ALL RILED UP ON A ROLL. AND IT'S YOUR
16	MEETING.
17	CHAIRMAN FISCHER-COLBRIE: NO, NO. ROSA,
18	I THINK YOU'RE DOING A GREAT JOB ON THE
19	FACILITATION. SO LET'S CONTINUE WITH YOU FIELDING
20	THE QUESTIONS BECAUSE I THINK THAT'S MOST
21	APPROPRIATE. AND I'M NOT SURE WHO WAS NEXT UP.
22	MAYBE FRED OR KEITH. I'M NOT SURE.
23	DR. FISHER: I'M HAPPY TO DEFER TO KEITH.
24	DR. YAMAMOTO: NO. FRED, YOU'RE UP. GO
25	AHEAD.

1	CHAIRMAN FISCHER-COLBRIE: GO AHEAD. AND,
2	ROSA, YOU GO AHEAD AND FLAG WHO'S UP NEXT AND WHO TO
3	CALL ON BECAUSE YOU'RE DOING A GREAT JOB. SO THANK
4	YOU.
5	DR. FISHER: SO APOLOGIES FOR MY CAMERA.
6	MY COMPUTER HAS DECIDED TO TELL ME TO LOOK FOR
7	CAMERA THAT DOESN'T EXIST.
8	IT'S BEEN A QUESTION REALLY JUST TRYING TO
9	UNDERSTAND WHEN WE TALK ABOUT INDUSTRY PARTNERSHIPS,
10	WHAT EXACTLY DO WE MEAN BY INDUSTRY?
11	NOTWITHSTANDING PAT'S RECENT COMMENT, IT OCCURS TO
12	ME THAT A SIGNIFICANT PORTION OF THE GRANTS THAT WE
13	FUND ARE WITH SMALL BIOTECHS. WHEN I THINK OF
14	INDUSTRY, I THINK ABOUT MID TO LARGE CAP BIOTECHS
15	AND PHARMA. SO WHEN WE TALK ABOUT INDUSTRY, ARE WE
16	INCLUDING THE ONE- OR TWO-PERSON SHOPS IN A
17	FOR-PROFIT START-UP AS INDUSTRY? WHERE IS THE
18	CUTOFF, IF ANY, IN TERMS OF HOW YOU DEFINE INDUSTRY?
19	DR. CANET-AVILES: THAT'S A VERY GOOD,
20	APPROPRIATE QUESTION BECAUSE LIKE IF WE THINK
21	ABOUT SO TWO THINGS. ONE IS THE INDUSTRY FOR
22	GOAL 1 AND THE OTHER IS THE INDUSTRY FOR GOAL 2. I
23	THINK THAT THOSE COULD BE TWO DIFFERENT.
24	SO WHEN WE TALK ABOUT GOAL 2, THE ROLE OF
25	THE INDUSTRY PARTICIPATION COULD BE MORE ON

1	REFINING, SCALING, AND COMMERCIALIZING THOSE
2	TECHNOLOGIES, DEVELOPING THE ACADEMIC SETTINGS. AND
3	THEY COULD PROVIDE EXPERTISE IN CLINICAL
4	APPLICATION, REGULATORY COMPLIANCE, MARKET
5	READINESS. SO FOR THAT IT'S NOT A SMALL SHOP. THAT
6	WOULD BE MY AND AS I SAID, WE'RE STILL DEFINING
7	ALL OF THIS. OH, SHYAM, DO YOU WANT TO ANSWER THE
8	QUESTION? HE HAS THE HAND. GO AHEAD.
9	DR. PATEL: THANK YOU, ROSA. THAT IS A
10	VERY GOOD QUESTION, AND I THINK WE TEND TO LUMP A
11	LOT OF DIFFERENT TYPES OF INDUSTRY PLAYERS INTO THIS
12	CONVERSATION. SO I THINK, AS ROSA HAS APPROPRIATELY
13	INDICATED, IT WOULD BE DIFFERENT DEPENDING ON THE
14	TWO GOALS THAT ARE ESTABLISHED HERE.
15	SO IF YOU DON'T MIND, I'LL JUST WALK
16	THROUGH A COUPLE OF QUICK EXAMPLES. SO FOR GOAL 1,
17	WHEN WE'RE TALKING ABOUT BIOMARKERS AND TARGETS, THE
18	KEY THING IS GOING TO BE TO ENGAGE THE BIGGER
19	BIOTECH AND PHARMA COMPANIES THAT YOU'RE MENTIONING,
20	THE MID CAPS AND THE LARGE CAPS. AND AS ROSA
21	APPROPRIATELY POINTED OUT, IT'S GOING TO BE GETTING
22	AWAY FROM A CAPITAL COMMITMENT TO BEING MORE OF A
23	RESOURCE AS WELL AS ADVISORY COMMITMENT TO GET TO
24	THE AREAS THAT PAT AND OTHERS HAVE POINTED OUT WITH
25	RESPECT TO REPRODUCIBILITY, STUDY DESIGN,

1	SCALABILITY, EVEN HAVING ACCESS TO HIGH THROUGHPUT
2	SCREENING MECHANISMS MIGHT BE USEFUL THAT SOME OF
3	THE PLAYERS CAN PROVIDE. SO IT'S TO THAT LEVEL.
4	AND ROSA OBVIOUSLY HAS A LOT OF EXPERIENCE
5	IN THAT FROM NIH AMP FNIH AMP ACTIVITIES. AND AS
6	YOU ALL KNOW, THERE ARE A LOT OF PARTNERSHIPS THAT
7	PHARMA WILL ENGAGE WITH ON AN INDIVIDUAL LEVEL WITH
8	EARTH PI'S DIRECTLY OR WITH ACADEMIC INSTITUTIONS TO
9	A BROADER LEVEL WHERE THEY GET A VIEW INTO DATA OR
10	IP AND SO ON. SO FOR US IT'S GOING TO BE
11	INCENTIVIZING THOSE LARGER COMPANIES TO ENGAGE WITH
12	US ON THESE AREAS WHERE THERE MAY BE A WIN-WIN FOR
13	ALL SIDES.
14	FOR THE TECHNOLOGY PLATFORM SIDE, THERE
15	ARE DIFFERENT TYPES OF PLAYERS THAT COULD BE
16	RELEVANT HERE. YOU COULD HAVE A SMALL TECHNOLOGY
17	INNOVATOR THAT IS DEVELOPING A NEW TECHNOLOGY, BUT
18	WOULD REALLY BENEFIT FROM ACADEMIC COLLABORATIONS TO
19	HELP REFINE THAT TECHNOLOGY AND MAKE IT MORE
20	APPLICABLE FOR THEIR DEVELOPMENT. A LOT OF THE
21	TIMES THESE TYPES OF COMPANIES FOSILS ON SHAPTNS WITH
	TIMES THESE TYPES OF COMPANIES FOCUS ON SHARING WITH
22	LARGER BIOPHARMA PARTNERS. AND THEY DON'T RECOGNIZE
23	LARGER BIOPHARMA PARTNERS. AND THEY DON'T RECOGNIZE
<ul><li>22</li><li>23</li><li>24</li><li>25</li></ul>	LARGER BIOPHARMA PARTNERS. AND THEY DON'T RECOGNIZE THAT A LOT OF ACADEMIC INVESTIGATORS ARE ACTUALLY

1	THEME THAT I AND MY TEAM AS WELL AS CIRM AS A WHOLE
2	HAS BEEN PROPAGATING ACROSS ALL OF OUR INDUSTRY
3	OUTREACHES, THAT THERE'S A LOT OF CLINICAL
4	DEVELOPMENT HAPPENING IN THE ACADEMIC SPACE. AND
5	THAT'S WHERE YOU CAN PARTNER WITH THEM.
6	AS ROSA APPROPRIATELY MENTIONED, FOR THE
7	LARGER, THE MID CAP AND LARGE CAP BIOPHARMA
8	COMPANIES, ON THE TECHNOLOGY DEVELOPMENT, IT'S
9	REALLY IDENTIFYING THE NEEDS. WHAT ARE THE NEEDS?
10	WHAT RESOURCES CAN THEY SHARE? AND THEN HOW CAN
11	THEY TAKE WHAT IS BEING DEVELOPED AND UTILIZE IT FOR
12	THERAPEUTIC DEVELOPMENT? SO I THINK IT DEPENDS, BUT
13	WE DO NEED TO HAVE FLEXIBILITY AND COMPARTMENTALIZED
14	DIFFERENT TYPES OF INDUSTRY PLAYERS AND BRING THEM
15	TO THE FOLD IF WE'RE GOING TO DO THIS APPROPRIATELY
16	AND EFFECTIVELY GOING FORWARD.
17	DR. FISHER: THANK YOU FOR THAT. MY
18	HOPE I THINK A LOT OF INNOVATION COMES FROM SMALL
19	BIOTECHS THAT HAVE A THERAPEUTIC IDEA THAT NEEDS TO
20	BE PURSUED. AND I HOPE WE FIND A WAY TO CONTINUE TO
21	INCLUDE THOSE FOLKS IN OUR DISCOVERY AND POTENTIALLY
22	CLIN PROJECTS.
23	DR. CANET-AVILES: ABSOLUTELY. YEAH.
24	THANK YOU, SHYAM. THAT WAS EXCELLENT. APPRECIATE
25	IT.

1	YES. THE ANSWER TO YOU, FRED, IS YES.
2	AND WE STILL HAVE NOT GONE THROUGH GOALS 3 AND 4.
3	AND I THINK GOAL 4 WILL BE TALKING MORE ABOUT WHAT
4	YOU ARE REFERRING. SO WE WILL DISCUSS IT IN AUGUST,
5	BUT THANK YOU.
6	KEITH.
7	DR. YAMAMOTO: TERRIFIC. ROSA, I'LL JUST
8	UNDERSCORE WHAT OTHERS HAVE SAID. AND THANK YOU FOR
9	A FANTASTIC PRESENTATION BACKED BY AN ENORMOUS AND
10	COMPREHENSIVE BODY OF WORK BY YOUR TEAM. SO
11	CONGRATULATIONS TO YOU ALL AND THANKS TO YOU ALL.
12	FANTASTIC.
13	THREE QUICK POINTS OR QUESTIONS, I GUESS.
14	YOU PRESENTED A VERY COMPREHENSIVE SUMMARY OF
15	CRITERIA TO EXAMINE IN EVENTUALLY MAKING A CHOICE OF
16	WHERE TO PUT FOCUS, WHETHER THERE ARE GOOD EXISTING
17	STEM CELL MODELS, WHETHER THERE'S A HIGH NEED FOR
18	BIOMARKERS, THE NIH SPEND, THE ECONOMIC BARRIER IN
19	CALIFORNIA ECONOMIC BURDEN IN CALIFORNIA, AND SO
20	FORTH. A LOT OF CRITERIA.
21	I THINK I KNOW THE ANSWER TO THIS, BUT
22	I'LL JUST ASK IT SO YOU CAN COMMENT. AND THAT IS
23	HOW ARE YOU GOING TO MAKE A DECISION FOR ANY GIVEN
24	DISEASE? THE OVERLAP OF CRITERIA ARE NOT SIMPLE,
25	AND DOESN'T SIMPLY LINE UP THAT ONE DISEASE JUMPS

1	OUT BECAUSE THERE'S A GREAT NEED IN EACH OF THE
2	CRITERIA AREAS. AND SO YOU ARE GOING TO HAVE HARD
3	DECISIONS TO MAKE.
4	HAVE YOU THOUGHT ABOUT HOW THAT WILL BE
5	DONE, CERTAINLY NOT FORMULAICALLY, BUT HAVE YOU
6	THOUGHT ABOUT HOW YOU WILL BE MAKING THOSE CHOICES?
7	SO THAT'S THE FIRST QUESTION.
8	AND THEN A COMMENT THAT EXPANDS ON OR
9	MAYBE UNDERSCORES WHAT PAT HAS BEEN TALKING ABOUT.
10	YOU REALLY MADE THE POINT STRONGLY AT THE BEGINNING
11	THAT IT WOULD BE USEFUL TO THINK ABOUT ESTABLISHMENT
12	OF A DATA COORDINATING AND MANAGEMENT CENTER AS A
13	COMPUTATIONAL PROBLEM AND A DATA SCIENCE PROBLEM.
14	IT'S A SUBSTANTIAL ONE BECAUSE, IN FACT, YOU'RE NOT
15	JUST TRYING TO PUT EVERYTHING ONTO ALL THIS
16	INFORMATION ONTO A GRID. YOU'RE TRYING TO
17	UNDERSTAND THE INTERACTIONS AND RELATIONSHIPS
18	BETWEEN DIFFERENT DATA TYPES. AND THAT IS A
19	SUBSTANTIAL CHALLENGE. AND I THINK PAT'S IDEA THAT
20	HE VOICED OF REALLY HAVING A SECTOR OF OFFERINGS FOR
21	FUNDING FOR REALLY DATA SCIENCE VERY MUCH IN THE
22	CONTEXT OF BUILDING A KNOWLEDGE NETWORK, SOMETHING
23	THAT WE'VE TALKED ABOUT IN THIS ORGANIZATION BEFORE
24	WHEN WE INTERACTED WITH THE PRECISION MEDICINE
25	INITIATIVE WHICH HAD THIS NOTION OF BUILDING A
	40

1	KNOWLEDGE NETWORK AT ITS CORE.
2	SO INTERACTING WITH GROUPS THAT ARE ALONG
3	THE WAY ON THAT. UCSF HAS MADE A LOT OF PROGRESS IN
4	THIS REALM, BUT WE'RE CERTAINLY NOT THE ONLY ONES,
5	COULD BE USEFUL. AND THEN MORE BROADLY, OPENING UP
6	THE POTENTIAL FOR FUNDING FOR DATA SCIENTISTS WHO
7	ARE ALREADY THINKING ABOUT THESE CHALLENGES, I
8	THINK, COULD BE VERY PRODUCTIVE. AND I THINK THAT
9	THAT'S PERFECT.
10	AND THEN FINALLY, JUST TO COMMENT ON WHERE
11	WE ARE AT THE NATIONAL LEVEL, I'M ACTUALLY REACHING
12	YOU ALL FROM DC. I'M IN TOWN TO IN FACT, I JUST
13	HAD A LONG MEETING WITH BILL CASSIDY, WHO YOU KNOW
14	IS THE RANKING MEMBER ON THE SENATE HEALTH COMMITTEE
15	WHICH OVERSEES THE SENATE SIDE, AUTHORIZATION
16	COMMITTEE FOR THE NIH. AND BILL HAS BEEN THINKING
17	FOR A YEAR AND A HALF. I'VE BEEN TALKING A LOT WITH
18	HIM ABOUT A COMPREHENSIVE REAUTHORIZATION OF THE NIH
19	THAT HASN'T BEEN DONE SINCE 2006 WHEN JOE BARTON ON
20	THE HOUSE SIDE LAUNCHED A VERY EFFECTIVE AND HELPFUL
21	REAUTHORIZATION. I THINK THAT CASSIDY IS THINKING
22	SIMILARLY IN THIS WAY.
23	HE HAS RELEASED A DOCUMENT ACTUALLY BEFORE
24	THE CATHY MCMORRIS-RODGERS DOCUMENT SUMMARIZING THE
25	INPUTS THAT HE RECEIVED FROM HIS RPE ON NIH REFORM.

1	AND I THINK THAT'S MOVING FORWARD WELL. SO I'M
2	HOPING THAT THE KIND OF STRATEGIES AND THOUGHTS THAT
3	HE HAS HAD IN LOOKING AT NIH REFORM CAN CARRY THE
4	DAY AT THE END OF THE DAY.
5	THE MCMORRIS-RODGERS DOCUMENT IS
6	INCREDIBLY DESTRUCTIVE. IT IS REALLY WRITTEN FROM
7	THE STANDPOINT THAT REFLECTS MCMORRIS-RODGERS' ANGER
8	AT NIH EVER SINCE WUHAN. HER VIEW IS THAT THE SARS
9	COV2 VIRUS WAS A LAB ESCAPEE FROM WUHAN. AND THEN
10	MOST DAMAGINGLY WAS PARTIALLY FUNDED BY THE
11	ECO-HEALTH ALLIANCE SUBCONTRACT THAT WAS LET TO
12	WUHAN VIROLOGY.
13	I THINK THERE'S ESSENTIALLY NO DATA THAT
14	ARE CONSISTENT WITH THOSE NOTIONS, BUT IT'S A
15	PREVALENT VIEW ON THE HOUSE ENERGY AND COMMERCE
16	COMMITTEE LEADERSHIP. AND IT LED TO THE REALLY
17	DESTRUCTIVE DOCUMENT THAT SHE'S PUT FORTH. I DON'T
18	THINK THAT WILL GO FORWARD. I AGREE WITH PAT. THE
19	WORD ON THE STREET IS THAT IT WON'T, AND I'M HOPING
20	THAT IT WILL BE MODERATED, TO PUT IT MILDLY, BY THE
21	KINDS OF THINGS THAT CASSIDY IS THINKING ABOUT.
22	SO I THINK WE NEED TO JUST STAY ALERT.
23	BUT THE BOTTOM LINE IS THAT THE BOTTOM LINE FOR
24	CIRM IS THAT WE NEED TO BE ALWAYS THINKING ABOUT
25	WHERE CIRM CAN MAKE UNIQUE ATTRIBUTIONS IN THE AREA

1	OF RESEARCH OF ITS CHOOSING. AND A PART OF THAT
2	DECISION HAS GOT TO LOOK AT WHAT THE POSSIBLE
3	OVERLAPS ARE FOR THE NIH. IF THAT IS SOMETHING
4	THAT'S GOING TO BE CHANGING SOON, WELL, WE'LL JUST
5	HAVE TO BE ALERT TO IT. I ACTUALLY DON'T THINK THAT
6	WE'LL KNOW THE ANSWER TO THAT ON NOVEMBER 4TH. I
7	DON'T THINK THE MCMORRIS-RODGERS STRATEGY IS
8	ACTUALLY GOING TO MOVE FORWARD IN THIS CONGRESS.
9	AND SO IT'S GOING TO BE AFTER THAT THAT THE TWO
10	HOUSES OF CONGRESS GET DOWN TO BUSINESS OF WRITING
11	OF AUTHORIZING LANGUAGE FOR THE NIH THAT WE'LL BEGIN
12	TO SEE HOW THIS IS ALL GOING TO BREAK OUT.
13	SO SIMPLY STAYING ALERT AND ON TOP OF THE
14	SITUATION. AND I AND OTHERS ARE IN TOWN WORKING ON
15	THIS VERY PROBLEM. SO I'LL CERTAINLY KEEP ALL OF
16	YOU ADVISED AS EXPEDITIOUSLY AS POSSIBLE.
17	DR. LEVITT: IF I CAN FOLLOW UP BRIEFLY,
18	TAKE THE CO-CHAIR PREROGATIVE. AS KEITH WAS TALKING
19	ABOUT THE DATA SCIENCE THING, I WAS THINKING WE HAVE
20	IN THE REMIND PROGRAMS, WE HAVE A REQUIREMENT
21	FOR I THINK WE HAVE A REQUIREMENT FOR DATA
22	SCIENCE OR INFORMATICS AS PART OF THE BIOMEDICAL
23	RESEARCH TEAM. IF YOU JUST FLIP THAT YOU HAVE A
24	TEAM OF DATA SCIENTISTS AND THEN YOU HAVE A
25	REQUIREMENT FOR A BIOMEDICAL RESEARCHER TO BE PART

1	OF THAT TEAM THAT BASICALLY DOES THE SAME THING,
2	MEANING THAT THERE WILL BE INPUT FROM THE VERY
3	BEGINNING FROM SOMEBODY WHO UNDERSTANDS THE
4	BIOLOGICAL CONSTRUCT, THE DISEASE CONSTRUCTS, WHICH
5	CAN REALLY BE IMPORTANT SO THEY'RE NOT OUT THERE ON
6	THEIR OWN AS DATA SCIENTISTS. SOME ARE GREAT AND
7	UNDERSTAND IT; OTHERS REALLY HAVE CHALLENGES. SO
8	THEY DO A TON OF ANALYSES, AND THEN YOU END UP
9	SCRATCHING YOUR HEAD BECAUSE THEY KIND OF MISSED THE
10	BOAT ABOUT THE
11	DR. YAMAMOTO: THAT'S RIGHT.
12	DR. LEVITT: ABOUT THE BIOLOGY. SO IF
13	YOU HAVE THAT JUST REVERSE REQUIREMENT, IT WOULD
14	REALLY IT WOULD BE VERY EXCITING BECAUSE I DON'T
15	KNOW OF OTHER INITIATIVES THAT DO THAT. IT WOULD BE
16	VERY, VERY COOL.
17	AND THE OTHER THING I JUST WANTED TO
18	MENTION IS I KNOW YOU'RE GOING TO DO THIS WITH THE
19	RFA'S, BUT DEFINING WHAT WE MEAN BY, LIKE, THE
20	ACTUAL LIKE THERE'S SO MANY WAYS TO DEFINE
21	PARTNERSHIPS AND RELATIONSHIPS. DEFINING THOSE
22	REALLY SPECIFICALLY, AS SHYAM WAS SPEAKING, IT JUST
23	SORT OF TRIGGERED IN MY BRAIN, OKAY, SO WHAT ARE
24	GOING TO BE IN TALKING TO THE POTENTIAL INDUSTRY
25	PARTNERS, WHAT DO THEY FEEL IS GOING TO BE THE MOST

1	PRODUCTIVE WAY OF DEFINING A PARTNERSHIP, NOT JUST
2	US DEFINING IT AND TELLING THEM HERE'S THE WAY YOU
3	HAVE TO PARTNER WITH THE ACADEMICS, BUT GETTING
4	INPUT FROM THEM EARLY ON BEFORE THE RFA EVEN COMES
5	OUT AND SAYING HERE'S THE DEFINITION OF WHAT WE MEAN
6	BY A PARTNERSHIP. AND I THINK THAT WOULD REALLY
7	ALSO SAVE A LOT OF TIME. ANYWAY, YEAH.
8	DR. CANET-AVILES: WE WILL DEFINITELY TAKE
9	THOSE THAT INPUT, THAT FEEDBACK, WHICH IS
10	EXCELLENT, INTO ACCOUNT AS WE DEVELOP THE CONCEPT,
11	IF APPROVED. RIGHT? BECAUSE FROM EXPERIENCE AT THE
12	FNIH LEVEL, YOU GET WHAT THE INDUSTRY PARTNERS
13	WANTED WAS THE DEPTH OF THE DATA WAS THAT GENERATED
14	THE FUNDING FROM THE NIH, RIGHT, AND THE ACCESS TO
15	ALL THESE MULTIPLE ACADEMIC AND VALIDATION, THE
16	REPRODUCIBILITY, AND THEY BRINGING EXPERIENCE IN
17	TARGET VALIDATION, RIGHT, AT THE PRECOMPETITIVE
18	LEVEL. THIS COULD MESH WITH THE COMPETITIVENESS,
19	RIGHT. SO WE NEED TO TALK TO THEM AND FIGURE OUT
20	WHAT IS IT THAT COULD MAKE THE DEAL FOR THEM, AND
21	THAT COULD ALSO PLEASE ACADEMICS BECAUSE EVERYBODY
22	NEEDS TO AT THE END OF THE DAY BE HAPPY.
23	NOW, WE DO HAVE THE FUNDING WHICH IS WHAT
24	WE ARE OFFERING AND THE PATIENTS RIGHT THERE WAITING
25	FOR SOLUTIONS.

1	THERE WAS A QUESTION THAT SO THANK YOU,
2	KEITH, FOR BEING IN DC FOR ALL OF US ON BEHALF OF
3	ALL OF US. YOU ASKED ABOUT THE CRITERIA TO EXAMINE
4	WHERE TO PUT THE FOCUS. HOW WOULD WE MAKE A
5	DECISION? AND THERE ARE DIFFERENT WAYS TO MAKE A
6	DECISION. WE COULD THEN SELECT DISEASES. WHAT WE
7	WOULD ASK IS, THROUGH THE ELIGIBILITY CRITERIA, WE
8	COULD BE ASKING THAT IF YOU ARE COMING TO IF
9	THERE IS AN APPLICATION, THE DISEASE HAS TO HAVE A
10	VALIDATED CELL MODEL. THE NEED FOR BIOMARKERS NEEDS
11	TO BE X. RIGHT. IT HAS TO BE. AND YOU NEED TO
12	HAVE A PARTNERSHIP, ONCE WE DEFINE THE TERMS OF THE
13	PARTNERSHIP, WITH INDUSTRY COMPONENTS. THAT
14	PARTNERSHIP NEEDS TO BE WITH SOMEBODY WHO HAS A
15	FOCUS IN THAT DISEASE, FOR EXAMPLE, OR THAT THE
16	NEEDS OF THE PROJECT ARE BEING CORROBORATED BY THE
17	INDUSTRY PART.
18	SO WE WOULD HAVE TO FIGURE OUT THOSE
19	DIFFERENT ASPECTS.
20	I HAVE ONE COMMENT ON THE DATA SCIENCE
21	PROBLEM. I SEE I THINK HAVING A DATA
22	COORDINATING MANAGEMENT CENTER, HAVING A KNOWLEDGE
23	PLATFORM WILL LEAD TO THAT PHASE OF HAVING THE DATA
24	SCIENCE AND HAVING POTENTIALLY A GROUP OF DATA
25	SCIENTISTS.

1	ANOTHER WAY TO APPROACH THIS IS HOW THE
2	NIH HAS DONE IT FOR SOME AMPS IS TO HAVE A
3	HACKATHON. TO HAVE LIKE A BUNCH OF DATA, SAY WE
4	GENERATED DATA THROUGH THIS SPECIFIC DISEASE, AND
5	THEN YOU HAVE ALL THESE DATA SCIENTISTS WITH SOME
6	BIOLOGISTS OR CLINICIANS THAT ARE COLLABORATING
7	TOGETHER TO SOLVE A SPECIFIC ISSUE. THAT'S ANOTHER
8	WAY TO APPROACH IT.
9	BUT THOSE ARE VERY GOOD IDEAS, AND WE WILL
10	TAKE THEM INTO ACCOUNT IF WE DEVELOP THE CONCEPT.
11	THANK YOU. ANYTHING ELSE? ANYBODY ELSE? I THINK
12	THE ROOM HAS J.T.
13	DR. THOMAS: YES. THIS IS IN RESPONSE TO
14	KEITH'S COMMENTS WHICH STARTED SORT OF WITH SHLOMO
15	AND PAT. I AGREE, KEITH. IT'S WONDERFUL YOU'RE
16	BACK THERE AND GIVING DIRECT INPUT HERE. THIS IS A
17	PRECARIOUS TIME. IT REQUIRES GREAT INSIGHT. AND
18	WE'RE ALL THE BETTER OFF FOR YOU BEING THERE TO HAVE
19	THESE DIRECT CONVERSATIONS.
20	WITH RESPECT TO CIRM BEING NIMBLE. AND
21	PERHAPS HAVING TO ADAPT WHETHER OR NOT THIS
22	PARTICULAR LEGISLATION GOES FORWARD, OF COURSE, CIRM
23	IS, AS IT WAS FORMED ORIGINALLY, WAS MEANT TO BE
24	SOMETHING THAT SUPPLEMENTED WHAT NIH WASN'T DOING.
25	AND THROUGHOUT OUR LIFE SPAN, WE HAVE EVALUATED SORT

1	OF WHERE WE ARE WITH RESPECT TO NIH FUNDING, HOW
2	BEST TO FILL GAPS WHERE FUNDING ISN'T GETTING DONE,
3	ET CETERA. SO WE HAVE A LONG-STANDING TRADITION AND
4	CULTURE OF ADAPTING TO THE NATIONAL FUNDING
5	FRAMEWORK.
6	SO TO THE EXTENT THAT ANYTHING DOES ARISE
7	OUT OF DRACONIAN LEGISLATION EITHER THROUGH CONGRESS
8	OR DEPENDING ON HOW THE ELECTION GOES, THE DIRECTION
9	IF THERE IS A NEW ADMINISTRATION, ET CETERA, THAT
10	DRUMS UP SUPPORT FOR REDUCING NIH FUNDING FOR
11	WHATEVER, WE ARE VERY OUR ANTENNA ARE ALWAYS UP.
12	AND WE WILL ADAPT ACCORDINGLY TO ANY SUCH ADVERSE
13	DEVELOPMENTS. SO THANK YOU ALL FOR THOSE COMMENTS.
14	THOSE ARE VERY IMPORTANT.
15	CHAIRMAN FISCHER-COLBRIE: ARE THERE OTHER
16	COMMENTS BEFORE WE OPEN FOR ANY POTENTIAL PUBLIC
17	COMMENTS THAT MIGHT BE THERE? ANY OTHER DISCUSSION
18	POINT PEOPLE WOULD LIKE TO BRING UP?
19	ROSA, I JUST WANT TO AMPLIFY AND ECHO THE
20	COMMENTS ABOUT THE DEPTH OF THE WORK AND WHAT'S BEEN
21	COMPLETED SO FAR. EXCITED ABOUT CONTINUING THE
22	ONGOING PROCESS LEADING TO THE GOALS AND ACKNOWLEDGE
23	THAT WE HAVE STILL SIGNIFICANT WORK TO MOVE FORWARD
24	ON. BUT YOU HAVE COLLECTIVELY PROVIDED VERY
25	THOUGHTFUL DATA, INFORMATION, AND CONSIDERATION

1	AROUND FACTORS FOR US TO REVIEW AND COMMENT ON AS WE
2	GO FORWARD. AGAIN, JUST AMPLIFYING THE KUDOS TO THE
3	GROUP FOR THE DEPTH AND THE CONSIDERATION HERE.
4	IT'S EXTREMELY REMARKABLE. SO THANK YOU.
5	WITH THAT, ANY PUBLIC COMMENTS?
6	MR. TOCHER: WE'RE SURVEYING THE ROOM AND
7	ONLINE, AND IT DOES NOT APPEAR THAT WE HAVE ANY
8	PUBLIC COMMENT AT THIS TIME, MARK.
9	CHAIRMAN FISCHER-COLBRIE: OKAY. UNLESS
10	THERE'S ANY OTHER COMMENTS, OR J.T., IF THERE ARE
11	ANY OTHER ADDITIONAL COMMENTS YOU WOULD LIKE TO MAKE
12	BEFORE WE CONCLUDE THE MEETING.
13	DR. THOMAS: NO. OTHER THAN TO RESTATE
14	WHAT EVERYBODY HAS SAID, WHICH IS THANK ROSA AND HER
15	TEAM WRIT LARGE. THERE ARE MANY, MANY PEOPLE HERE
16	WORKING ON THIS AND GIVING INPUT. AND I THINK THIS
17	IS ALL PART OF THE EFFORT THAT'S GOING TO BE DRIVING
18	TOWARDS A CRESCENDO IN SEPTEMBER WHERE THE BOARD
19	WILL BE WELL POSITIONED HAVING BEEN ABLE TO HEAR A
20	LOT OF THINGS ALONG THE WAY TO MAKE A STRATEGIC
21	DECISION ON THE DIRECTION THAT'S GOING TO GUIDE US
22	FOR YEARS TO COME.
23	SO, ROSA AND SHYAM AND EVERYBODY, THANK
24	YOU VERY MUCH FOR YOUR OUTSTANDING WORK. AND SARA
25	AND THOMAS WHO HAVE BEEN SO INSTRUMENTAL AS WELL.

1	SO THANK YOU VERY MUCH.
2	DR. CANET-AVILES: AND OUR NEW CO-CHAIRS
3	AND CHAIR, LIKE MARK AND CAROLYN AND PAT, THANK YOU.
4	CHAIRMAN FISCHER-COLBRIE: I THINK WITH
5	THAT WE CAN CONCLUDE THE MEETING. SO THANK YOU VERY
6	MUCH FOR YOUR TIME, AND I'LL LOOK FORWARD TO YOUR
7	THOUGHTFUL INPUT AS WE CONTINUE TO GO THROUGH THIS
8	PROCESS. SO THANK YOU.
9	(THE MEETING WAS THEN CONCLUDED AT 10:21 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 VIRTUAL PROCEEDINGS BEFORE THE SCIENCE SUBCOMMITTEE AND TASK FORCE ON NEUROSCIENCE AND MEDICINE OF THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON JULY 11, 2024, 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 133 HENNA COURT SANDPOINT, IDAHO (208) 920-3543