INDEPENDEN CALIFORNIA IN ORC	BEFORE THE NCE SUBCOMMITTEE OF THE CITIZENS' OVERSIGHT COMMITTEE TO THE STITUTE FOR REGENERATIVE MEDICINE GANIZED PURSUANT TO THE STEM CELL RESEARCH AND CURES ACT REGULAR MEETING
LOCATION:	VIA ZOOM
DATE:	MARCH 26, 2024 3 P.M.
REPORTER:	BETH C. DRAIN, CA CSR CSR. NO. 7152
FILE NO.:	2024-16

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1	MARCH 27, 2024; 3 P.M.
2	
3	CHAIRMAN GOLDSTEIN: OKAY. IT'S 3:01. I
4	THINK WE SHOULD JUST GO AHEAD AND GET GOING. SO LET
5	ME CALL THIS MEETING TO ORDER. THE FIRST OFFICIAL
6	ACT WILL BE ASKING CLAUDETTE TO CALL THE ROLL.
7	MS. MANDAC: THANK YOU, LARRY.
8	MARIA BONNEVILLE.
9	VICE CHAIR BONNEVILLE: PRESENT.
10	MS. MANDAC: DEBORAH DEAS. MARK
11	FISCHER-COLBRIE.
12	MR. FISCHER-COLBRIE: HERE.
13	MS. MANDAC: ELENA FLOWERS.
14	DR. FLOWERS: HERE.
15	MS. MANDAC: JUDY GASSON.
16	DR. GASSON: HERE.
17	MS. MANDAC: LARRY GOLDSTEIN.
18	CHAIRMAN GOLDSTEIN: YEAH, HERE.
19	MS. MANDAC: DAVID HIGGINS.
20	DR. HIGGINS: HERE.
21	MS. MANDAC: VITO IMBASCIANI.
22	CHAIRMAN IMBASCIANI: HERE.
23	MS. MANDAC: PAT LEVITT.
24	DR. LEVITT: HERE.
25	MS. MANDAC: SHLOMO MELMED.
	3

1	DR. MELMED: HERE.
2	MS. MANDAC: CHRISTINE MIASKOWSKI. KAROL
3	WATSON. KEITH YAMAMOTO.
4	LARRY, WE HAVE QUORUM. BACK TO YOU.
5	CHAIRMAN GOLDSTEIN: OKAY. I THINK THAT
6	WE HAVE ONE AND ONLY ONE OBJECTIVE TODAY AS FAR AS I
7	KNOW. SO NO BUDGET APPROVALS OR OTHER DEEP MATTERS
8	TO DISCUSS. AND SO I THINK I'M TURNING IT OVER TO
9	ROSA FOR A PRESENTATION OF THE STRATEGIC ALLOCATION
10	FRAMEWORK; IS THAT RIGHT?
11	DR. THOMAS: ACTUALLY, LARRY, I'M GOING TO
12	SAY A FEW WORDS BEFORE INTRODUCING ROSA TO GO
13	THROUGH THAT FOR THE BENEFIT OF ALL CONCERNED HERE.
14	SO AS YOU ALL WILL RECALL AT OUR JANUARY
15	MEETING, WE TALKED ABOUT HOW CIRM IS AT AN
16	INFLECTION POINT DUE TO A VARIETY OF CIRCUMSTANCES
17	THAT ALL COME TOGETHER AT THE SAME TIME, BE THEY
18	BUDGETARY, BE THEY A DRAMATIC INCREASE IN
19	APPLICATIONS OF GRANTS AT ALL LEVELS, BE IT THE
20	DEVELOPING AND EVER CHANGING TECHNOLOGY FIELD, ET
21	CETERA, WE FELT, AS YOU VOTED ON EARLIER THIS YEAR,
22	THAT AMONG OTHER THINGS WE NEEDED TO HAVE A PERIOD
23	OF WHAT WE CALL FLOW CONTROL, WHICH WAS TO TAKE A
24	FEW MONTHS OFF IN ACCEPTING CLIN GRANTS FOR
25	CONSIDERATION TO BE REVISITED AT THE JUNE BOARD

MEETING.

1

PART AND PARCEL OF THAT PLAN WAS THE NEED 2 THAT WE HAVE IDENTIFIED TO FIGURE OUT WITH THE MONEY 3 THAT WE HAVE LEFT AT CIRM FOR THE BALANCE OF PROP 14 4 THAT WE NEED TO FIGURE OUT EXACTLY WHERE WE WANT TO 5 6 SPEND THAT MONEY. AND TOWARDS THAT END, WE EMBARKED UPON, AT THE SCIENCE SUBCOMMITTEE'S REQUEST AND 7 APPROVAL, THE PROCESS OF REPRIORITIZING HOW WE WILL 8 9 ACCOMPLISH THAT GOAL.

SO THE IDEA OF THIS REPRIORITIZING, WHICH 10 IS THE FIRST TIME WE'VE DONE THIS IN 20 YEARS 11 ESSENTIALLY, WE'VE CERTAINLY HAD MAJOR OPERATIONAL 12 CHANGES, BUT FROM THE SUBSTANTIVE STANDPOINT ON HOW 13 14 WE WANT TO CONDUCT OURSELVES, THIS IS A BIG DEAL FOR SO IT'S A MULTI-MONTH PROCESS THAT WE ARE 15 US. UNDERTAKING, WHICH IS BEING LED BY A PRIORITIZATION 16 17 WORKING GROUP IN-HOUSE AS OVERSEEN BY THE SCIENCE SUBCOMMITTEE IN GENERAL AND SPECIFICALLY BY MARK 18 19 FISCHER-COLBRIE, WHO IS HEADING UP THE OVERSIGHT OF REPRIORITIZING FOR THE SCIENCE SUBCOMMITTEE AND THE 20 BOARD AS THINGS PROCEED ALONG. 21

THIS PROCESS IS SOMETHING THAT'S GOING TO
TAKE US THROUGH ON OUR CURRENT CALENDAR SEPTEMBER,
AT WHICH POINT WE LOOK TO HAVE RECOMMENDATIONS TO
THE BOARD ON HOW WE WOULD GO ABOUT SPENDING OUR

1	MONEY GOING FORWARD IN A VERY COMPREHENSIVE AND
2	WELL-THOUGHT OUT PLAN.
3	THE THING TO KEEP IN MIND HERE IS THAT,
4	BECAUSE WE ARE SHIFTING COURSE A BIT UNDER THE
5	CONTEXT OF THE STRATEGIC PLAN, THIS NEW WAY OF
6	ADVANCING THE STRATEGIC PLAN IS GOING TO BE
7	TARGETING HOW WE SPEND OUR MONEY THROUGH THE REST OF
8	THE LIFE OF PROP 14.
9	NOW, HAVING SAID THAT, OBVIOUSLY THERE
10	COULD BE TIMES IN THE INTERIM WHERE WE WILL WANT TO
11	REVISIT, AS WE ARE DOING HERE, TO FIGURE OUT IF
12	THERE IS A BETTER WAY AT THAT POINT IN TIME TO
13	PROCEED. BUT FROM THE STANDPOINT OF WHAT WE'RE
14	DOING TODAY, THE OBJECT IS TO SET COURSE FOR THE
15	BALANCE OF THE TIME THAT PROP 14 FUNDS ARE
16	AVAILABLE.
17	SO AS YOU MAY RECALL HERE INSIDE CIRM AND
18	INSIDE OUR PRIORITIZATION WORKING GROUP, WHICH, BY
19	THE WAY, YOU WILL BE INTERESTED TO KNOW HAS HAD A
20	NUMBER OF TWO- TO FOUR-HOUR MEETINGS THAT WE'VE SET
21	ASIDE WHERE WE'VE BEEN DISCUSSING THIS PROCESS AND
22	HOW WE'RE GOING TO GO ABOUT DOING THINGS, ROSA HAS
23	TAKEN THE LEAD IN-HOUSE TO PROJECT MANAGE THIS WHOLE
24	THING. AND THE ENTIRE REST OF THE WORKING GROUP HAS
25	PUT IN MANY, MANY HOURS. AND THE TEAM EFFORT I'D

1	LIKE TO REPORT TO YOU HAS BEEN EXCEPTIONAL,
2	COLLABORATIVE, HIGHLY SUPPORTIVE, ALL ON THE SAME
3	PAGE, AND DRIVING TOWARDS WHAT WE THINK WILL BE A
4	VERY GOOD RESULT FOR THE BOARD AND CIRM WRIT LARGE
5	AT THE END OF THE DAY IN SEPTEMBER WHEN YOU ALL
6	HOPEFULLY WILL VOTE ON WHAT TO DO AT THAT POINT.
7	SO TODAY'S MEETING AND THURSDAY'S BOARD
8	MEETING WILL BE, TODAY IN ITS ENTIRETY AND PART OF
9	THURSDAY'S BOARD MEETING WILL BE ABOUT THE PROCESS
10	THAT WE ARE PUTTING TOGETHER TO DRIVE TOWARDS
11	ULTIMATE RECOMMENDATIONS. THIS MEETING IS NOT MEANT
12	IN ANY WAY TO DISCUSS SUBSTANCE, SPECIFIC
13	PRIORITIES, SPECIFIC RECOMMENDATIONS, ANYTHING
14	BESIDES PROCESS. AND SO ROSA HAS PUT TOGETHER A
15	PRESENTATION FOR YOU ALL TO CONSIDER HERE ON
16	PROCESS. AND I THINK WITH THAT TURN IT OVER TO YOU.
17	DR. CANET-AVILES: THANK YOU, J.T. THANK
18	YOU FOR THE INTRODUCTION. CAN YOU ALL HEAR ME?
19	CHAIRMAN GOLDSTEIN: YES.
20	DR. CANET-AVILES: GREAT. FANTASTIC. SO
21	NEXT SLIDE, SARA.
22	SO ULTIMATELY THIS STRATEGIC
23	PRIORITIZATION FRAMEWORK THAT J.T. HAS SO WELL
24	INTRODUCED TO US IS A TESTAMENT TO CIRM'S PROFOUND
25	DEDICATION TO OUR MISSION, WHICH IS HERE, AND THAT
	7

1	ULTIMATELY LEADS TO TRANSFORMING HEALTHCARE THROUGH
2	THE POWER OF REGENERATIVE MEDICINE, INCLUDING STEM
3	CELLS AND GENETIC THERAPIES. NEXT SLIDE.
4	SO THIS IS THE STRATEGIC ALLOCATION
5	FRAMEWORK THAT WE ARE PRESENTING TODAY IS A
6	STRUCTURED DATA-DRIVEN APPROACH TO PRIORITIZE
7	RESOURCE ALLOCATION AND PROVIDE FURTHER GRANULARITY
8	IN TERMS OF GOALS AND THEIR SUCCESS MEASURES AS
9	WELL, THAT WILL ULTIMATELY LEAD TO RECOMMENDATIONS
10	FOR CONTINUED IMPLEMENTATION OF OUR STRATEGIC PLAN.
11	WHAT WE ARE SAYING HERE IS THAT WE ARE BUILDING ALL
12	THESE OVER THE BASIS OF A STRATEGIC PLAN, BUT
13	BASICALLY WE ARE PROVIDING A LOT MORE GRANULARITY
14	AND SUCCESS MEASURES TO FIGURE OUT HOW TO MOVE
15	FORWARD. NEXT SLIDE.
16	SO IN TERMS OF BACKGROUND, WHAT'S THE
17	BACKGROUND OF THIS STRATEGIC ALLOCATION FRAMEWORK?
18	THE FRAMEWORK HAS BEEN DEVELOPED OVER THE PAST
19	MONTH, AND IT'S POISED TO GUIDE CIRM IN MAKING
20	INFORMED DECISIONS REGARDING THE DISTRIBUTION OF OUR
21	REMAINING FUNDS. AS A PIONEER ENTITY IN THE REALMS
22	OF STEM CELL RESEARCH AND REGENERATIVE MEDICINE, OUR
23	LEGACY IS FOUNDED IN THE ADVANCEMENT OF SCIENTIFIC
24	DISCOVERY TOWARDS TANGIBLE MEDICAL BREAKTHROUGHS AND
25	ULTIMATELY CURES.

8

1	OUR INSTITUTE HAS BEEN INSTRUMENTAL, AS WE
2	ALL KNOW, IN FUNDING CUTTING EDGE RESEARCH,
3	DEVELOPING ROBUST INFRASTRUCTURE. WE'VE PIONEERED A
4	GREAT NETWORK OF EDUCATIONAL PROGRAMS, AND WE ARE
5	CATALYZING THE PROGRESSION FROM REGENERATIVE
6	MEDICINE RESEARCH TO CLINICAL PRACTICAL
7	APPLICATIONS, AND ULTIMATELY, HOPEFULLY, CURES.
8	AS WE WILL SEE IN THE NEXT SLIDE, THE
9	FIELD OF REGENERATIVE MEDICINE HAS GROWN
10	EXPONENTIALLY IN THE PAST 17 YEARS. AND WE ALSO
11	KNOW THAT WE HAVE FINITE RESOURCES. DURING THE
12	SEPTEMBER 2023 SCIENCE SUBCOMMITTEE, BOARD MEMBER
13	MARK FISCHER-COLBRIE KICKED OFF A PRIORITIZATION
14	DISCUSSION IN WHICH THE NEED FOR A STRATEGIC
15	ALLOCATION PLAN WAS INTRODUCED. WE CALLED IT
16	PRIORITIZATION, AND WE ARE NOW CALLING IT A
17	STRATEGIC ALLOCATION FRAMEWORK.
18	DURING THAT MEETING THE BOARD ASKED CIRM
19	STAFF TO DEVELOP AN APPROACH AND RECOMMENDATIONS FOR
20	PRIORITIZATION. SO WHAT WE'VE DONE OVER THE PAST
21	MONTH IS THAT WE HAVE DEVELOPED THIS FRAMEWORK TO
22	PROVIDE THE STRATEGIC RECOMMENDATIONS FOR FUNDING
23	ALLOCATION. AND THIS FRAMEWORK PRESENTED REFLECTS A
24	COMPREHENSIVE STRATEGY THAT WILL DEFINE THE
25	RATIONALE, THE OBJECTIVES, THE SCOPE, AND TIMELINE

1	NECESSARY TO ESTABLISH CIRM'S FUTURE COURSE OF
2	ACTION.
3	THIS STRATEGY SERVES AS A ROADMAP FOR CIRM
4	TO MAXIMIZE ITS IMPACT AND TO CONTINUE ITS PIVOTAL
5	ROLE OF TRANSFORMING HEALTHCARE THROUGH THE POWER OF
6	REGENERATIVE MEDICINE FOR CALIFORNIA AND ULTIMATELY
7	FOR THE WORLD. NEXT SLIDE.
8	THE STRATEGIC ALLOCATION FRAMEWORK, THIS
9	IS AN OVERVIEW OF WHAT THE STRATEGIC ALLOCATION
10	FRAMEWORK CONSISTS IN. IT'S BASICALLY, AS I
11	MENTIONED, AGAIN, AND I KEEP SAYING IT A FEW TIMES
12	MORE, IS A STRUCTURE AND BASICALLY AN APPROACH TO
13	PRIORITIZE OUR RESOURCE ALLOCATION AND PROVIDE
14	FURTHER GRANULARITY IN TERMS OF OUR IMPACT GOAL. WE
15	HAVE DEFINED WHAT ARE THE GOALS. WE ARE DEFINING
16	WHAT THE IMPACT GOALS THAT WE WANT FOR OUR MISSION
17	TO BE, AND HOW ARE WE GOING TO MEASURE THAT IMPACT.
18	AND THAT WILL ULTIMATELY LEAD TO THE RECOMMENDATIONS
19	FOR CONTINUED IMPLEMENTATION OF OUR STRATEGIC PLAN.
20	THE DOCUMENT THAT WE HAVE ATTACHED AS
21	MATERIALS FOR TODAY'S MEETING IS STRUCTURED AS
22	FOLLOWS. WE HAVE BACKGROUND AND RATIONALE. IT
23	TALKS ABOUT WHAT ARE THE REMAINING FUNDS WHICH WE
24	ARE GOING OVER. AND WHAT'S THE LANDSCAPE OF THE
25	REGENERATIVE MEDICINE FIELD. AND THEN IT PROVIDES

10

1	AN OVERVIEW OF WHAT HAS BEEN CIRM'S IMPACT TO DATE,
2	AND THEN THE PROCESS OF WHAT THE STRATEGIC
3	ALLOCATION FRAMEWORK IS, AND THEN A PROPOSED
4	TIMELINE OF WHEN COULD THESE RECOMMENDATIONS WHAT
5	ARE THE ACTIVITIES THAT ARE INVOLVED IN THE
6	ALLOCATION FRAMEWORK, AND THEN WHEN THE
7	RECOMMENDATIONS COULD BE BECOME AVAILABLE. AND THE
8	OUTPUT OF ALL OF THIS WILL BE RECOMMENDATIONS FOR
9	STRATEGIC PRIORITIES THAT YOU WILL SEE AT THE END.
10	AND AS J.T. MENTIONED, WE HOPE TO HAVE READY FOR
11	SEPTEMBER ICOC MEETING.
12	THE NEXT SLIDE, THIS SLIDE PROVIDES A
13	SNAPSHOT OF CIRM'S REMAINING FUNDS FOR THE STRATEGIC
14	ALLOCATION THAT WE ARE TALKING ABOUT. AS YOU CAN
15	SEE ON THE LEFT, THE TOTAL RESEARCH AUTHORITY FROM
16	PROP 14 AND PROP 71 TOGETHER IS \$7.64 BILLION. THE
17	NET OF OPERATIONAL AND COMPLIANCE OVERSIGHT COSTS
18	FROM THE ORIGINAL 8.5 BILLION INITIALLY ALLOCATED BY
19	PROPOSITION 71 AND 14 TOGETHER. SO YOU KNOW IT'S 3
20	BILLION PLUS 5.5 BILLION RESPECTIVELY.
21	IN TERMS OF CURRENT FUND ALLOCATION, CIRM
22	HAS A REMAINING BALANCE OF \$3.54 BILLION. AND THIS
23	BALANCE RESULTS FROM ACCOUNTING FOR ALL EXPENDED
24	SCHEDULED PAYMENTS AND APPROVED ALLOCATIONS TO DATE.
25	WITHIN THIS REMAINING BALANCE, SPECIFIC EARMARKS, AS
	11

1	WE KNOW, HAVE BEEN MADE TO ADDRESS CRITICAL AREAS OF
2	NEED. ONE IS NEURO. WE HAVE \$1.11 BILLION LEFT OF
3	NEURO. THE ORIGINAL BALANCE WAS 1.38 BILLION
4	BECAUSE WE HAVE TO ACCOUNT FOR ADMINISTRATIVE COST.
5	OF THE 1.38, WE HAVE 1.11 THAT ARE ACCOUNTED IN THE
6	3.64, BY THE WAY. AND ALSO ANOTHER ALLOCATION IS
7	THE 93.56 MILLION THAT HAS BEEN ALLOCATED TO
8	INITIATIVES THAT AIM TO IMPROVE ACCESS AND
9	AFFORDABILITY TO TREATMENTS DEVELOPED FROM OUR
10	RESEARCH.
11	THIS FINANCIAL SNAPSHOT SETS THE STAGE FOR
12	CIRM TO DELIBERATE ON STRATEGIC ALLOCATION DECISIONS
13	THAT WILL SHAPE THE FUTURE OF OUR FIELD, BALANCING
14	THE DRIVE FOR INNOVATION WITH IMPERATIVE FOR
15	TREATMENTS TO BE BOTH ACCESSIBLE AND AFFORDABLE.
16	NEXT SLIDE PROVIDES AN OVERVIEW OF THE
17	REGENERATIVE MEDICINE LANDSCAPE. AS YOU CAN SEE,
18	THE LANDSCAPE IS MARKED BY AN ACCELERATING PACE OF
19	SCIENTIFIC BREAKTHROUGHS AND RAPID EXPANSION. THE
20	DATA SHOWN, WHICH IS TAKEN FROM THE ALLIANCE FOR
21	REGENERATIVE MEDICINE ANNUAL DATA REPORT OF 2022 AND
22	THE AMERICAN SOCIETY OF GENE AND CELL THERAPY
23	QUARTERLY REPORT OF 2021. THIS DATA UNDERSCORES THE
24	EXPONENTIAL GROWTH WITNESSED IN THE SECTOR SINCE
25	2005, MARKING A TRAJECTORY OF ACCELERATED

12

1	ADVANCEMENT IN STEM CELL AND GENETIC THERAPIES.
2	THIS EXPANSION IS EVIDENT, AS WE CAN SEE,
3	ACROSS VARIOUS PARAMETERS. THERE IS AN INCREASE IN
4	THE NUMBER OF COMPANIES ENGAGED IN THESE THERAPIES,
5	AND THERE'S AN EXPANSION IN THE PORTFOLIO OF NEW
6	PRODUCTS. THERE'S A GROWING NUMBER OF ACADEMIC
7	FACULTY DEDICATED TO THIS RESEARCH, AND A ROBUST
8	PIPELINE OF RESEARCH PROJECTS AND CLINICAL TRIALS.
9	THE FIRST CHART ON THE LEFT SHOWS US THE
10	INCREASE IN PUBLICATIONS RELATED TO STEM CELLS, GENE
11	THERAPY, AND CELL THERAPY, WHICH IS A TESTAMENT TO
12	THE GROWING INTEREST AND ADVANCEMENTS IN THE FIELD.
13	AND THEN THE MIDDLE AND RIGHT GRAPHS SHOWS THE
14	EXPANSION OF PIPELINES FOR GENE THERAPIES AND
15	NON-GENETICALLY MODIFIED CELL THERAPIES
16	CORRESPONDINGLY.
17	EACH BAR IN THESE TWO RIGHT AND MIDDLE
18	GRAPHS REPRESENT A SNAPSHOT OUR PROGRAMS AND
19	REFLECTS NOT ONLY THE INITIATION OF CLINICAL AND
20	PHASE 1 TRIALS, BUT ALSO THE PROGRESSION TO MORE
21	ADVANCED STAGES OF CLINICAL TESTING. THE LANDSCAPE
22	BRINGS TO THE FOREFRONT A COMPELLING NARRATIVE,
23	WHICH IS THAT THE FIELD OF REGENERATIVE MEDICINE IS
24	NOT JUST GROWING, IT IS THRIVING AT A PACE THAT
25	REQUIRES STRATEGIC AND THOUGHTFUL ALLOCATION OF

1	FUNDS. AND WE SEARCH A FERTILE GROUND FOR DISCOVERY
2	AND DEVELOPMENT. THE IMPLICATIONS FOR HEALTHCARE
3	ARE IMMENSE, WHICH UNDERSCORES THE IMPORTANCE OF
4	CIRM'S ROLE IN STRATEGICALLY FUNDING THE MOST
5	PROMISING AVENUES OF RESEARCH THAT CAN TRANSLATE
6	INTO LIFE-ALTERING TREATMENTS FOR PATIENTS.
7	THE NEXT SLIDE SHOWS US THE IMPACT TO
8	DATE. THIS IS JUST TO PROVIDE A BIT OF CONTEXT OF
9	WHERE THE RECOMMENDATIONS ARE BEING MADE FROM.
10	CIRM'S IMPACT TO DATE HAS BEEN REALIZED THROUGH
11	STRATEGIC INVESTMENTS IN A WIDE ARRAY OF INITIATIVES
12	THAT INTEROPERATE TOGETHER TO REALIZE OUR MISSION.
13	UNTIL NOW CIRM HAS BEEN COMMITTED TO A MULTIFACETED
14	APPROACH THAT INCLUDES A FOCUS ON BOTH RARE AND
15	PREVALENT DISEASES. AND THERE ARE FOUR KEY AREAS OF
16	EMPHASIS IN OUR IMPACT.
17	THE FIRST ONE IS DEVELOPMENT OF CELL AND
18	GENE THERAPIES. CIRM IS CURRENTLY COMMITTED TO
19	ADVANCING CELL AND GENE THERAPIES FROM RESEARCH TO
20	MARKET THROUGH CIRM'S CLINICAL FUNDING MODEL AND
21	CLINICAL INFRASTRUCTURE, EMPHASIZING NOT ONLY
22	PATIENT ACCESS, BUT ALSO THE SCALING OF
23	MANUFACTURING PROCESS TO MEET THE CLINICAL DEMAND.
24	WE'RE ALSO EMPHASIZING, AND AS YOU SEE AS
25	AN EXAMPLE, THE REMIND PROGRAM WAS ONE OF THEM,
	14

COLLABORATIVE NETWORKS FOR DISCOVERY RESEARCH. WE
ARE FOSTERING COLLABORATIVE NETWORKS TO UNITE
MULTIDISCIPLINARY TEAMS AND LEVERAGE EXTERNAL
OPPORTUNITIES AND FUNDING AS WELL, ENHANCING OUR
UNDERSTANDING OF DISEASE MECHANISMS AND LEADING TO
THE DISCOVERY OF NOVEL TARGETS AND BIOMARKERS THAT
WILL PUSH THE BOUNDARIES IN TREATMENT DEVELOPMENT
FOR THESE DISEASES.
IN LINE WITH PROPOSITION 14, THE FIRST
CASE THAT WE HAVE PRIORITIZED IS IN THE DEVELOPMENT
OF TREATMENTS FOR NEUROLOGICAL CONDITIONS IN THESE
COLLABORATIVE NETWORKS.
WE HAVE ALSO INVESTED AND HAVE AN IMPACT
IN TRAINING AND WORKFORCE DEVELOPMENT. AS WE'VE
HEARD AND AS WE WILL HEAR AT THE ICOC MEETING AN
UPDATE ON THIS, CIRM'S EDUCATIONAL INITIATIVES HAVE
SET A NEW STANDARD FOR WORKFORCE DEVELOPMENT IN
REGENERATIVE MEDICINE, CREATING A COMPREHENSIVE
NETWORK THAT CONNECTS TRAINING WITH REAL-WORLD
APPLICATION, AND WE ARE ALSO INTERCONNECTING WITH
OUR DIFFERENT PROGRAMS.
ADVANCEMENTS IN REGENERATIVE MEDICINE
TECHNOLOGIES IS ANOTHER OF THE IMPACTS THAT WE CAN
TALK ABOUT BY SUPPORTING INITIATIVES WHICH ADVANCE
OUR UNDERSTANDING OF COMPLEX DISEASES. CIRM HAS
15

1	BEEN TRANSLATING SCIENTIFIC EXCELLENCE INTO
2	SUBSTANTIAL PATIENT BENEFITS. AND WE HAVE SUPPORTED
3	RESEARCH TO IMPROVE THE DEVELOPMENT OF TECHNOLOGIES,
4	SUCH CELL REPROGRAMMING, GENETIC THERAPEUTIC
5	SYSTEMS, ETC.
6	THIS IS HOW WE SEE OUR IMPACT MOVING
7	FORWARD. AND PART OF THE RECOMMENDATIONS WILL BE
8	REEVALUATING THESE AS PART OF THE PROCESS. SO ONE
9	OF THE THINGS THAT WE'VE DONE AND WE'LL MOVE NOW
10	INTO WHAT GOES INTO THE NEXT SLIDE IS THAT THE
11	IMPACT GOALS FOR CIRM ARE BEING REEVALUATED. WHAT
12	DO WE MEAN BY THAT?
13	THIS SLIDE SHOWS WHAT THE STRATEGIC
14	ALLOCATION FRAMEWORK PROCESS CONSISTS IN. AND THE
15	STRATEGIC ALLOCATION FRAMEWORK THAT WE ARE
16	DISCUSSING TODAY IS A STRUCTURED, FORWARD-THINKING
17	APPROACH. THE FIRST PART OF IT IS THE IMPACT GOALS.
18	OUR TEAM IS DEFINING WHAT ARE THE GOALS AND THE
19	IMPACT AND SUCCESS MEASURES AND METRICS THAT WE WILL
20	HAVE TO DELINEATE IN ORDER TO HAVE AN IMPACT IN THE
21	YEARS THAT WE HAVE WITH LEFT OF CIRM.
22	THE NEXT THING THAT WE ARE DEVELOPING IS
23	THE GUIDING QUESTIONS THAT WILL HELP US FIGURE OUT
24	HOW ARE WE GOING TO LEAD TO THIS IMPACT. WHAT
25	RECOMMENDATIONS WE WILL HAVE TO MAKE TO THE BOARD IN
	16

1	ORDER TO LEAD TO THESE IMPACT GOALS.
2	AND THE THIRD POINT IS THE DATA COLLECTION
3	AND ANALYSIS. ONCE WE HAVE THESE GUIDING QUESTIONS,
4	OUR TEAM IS GOING TO DELINEATE WHAT DATA WE NEED TO
5	COLLECT AND ANALYZE IN ORDER TO ANSWER THE GUIDING
6	QUESTIONS THAT WILL HELP US MAKE RECOMMENDATIONS TO
7	LEAD TO THESE IMPACT GOALS.
8	SO THIS IS IN A NUTSHELL A VERY COMPLEX
9	PROCESS THAT WE HAVE UNDERTAKEN. RIGHT NOW WE ARE
10	IN THE SECOND STEP. WE'VE DEFINED THE IMPACT GOALS.
11	WE ARE DELINEATING THE GUIDING QUESTIONS THAT WILL
12	SHORTLY GO INTO THE DATA COLLECTION AND ANALYSIS,
13	WHICH IS WHAT WILL PROBABLY TAKE MOST OF THE TIME.
14	NEXT SLIDE SHOWS THE DESIGN QUESTIONS.
15	THESE ARE THE VERY, VERY HIGH LEVEL QUESTIONS THAT
16	WE CAME FROM IN TERMS OF THE STRATEGIC ALLOCATION
17	FRAMEWORK. HOW CAN CIRM MAKE THE GREATEST IMPACT ON
18	ITS MISSION, AND THAT'S HOW WE LED TO THIS IMPACT
19	GOAL DEFINITION. FIRST WE NEED TO DEFINE THAT AND
20	PROVIDE SUCCESS MEASURES TO BE ABLE TO MEASURE THAT
21	WE GOT THOSE GOALS. AND HOW MIGHT CIRM EFFECTIVELY
22	ALLOCATE THE REMAINING BUDGET OF THIS \$3.54 BILLION
23	TO MAKE THAT IMPACT ON OUR MISSION? RIGHT.
24	SO THAT'S KIND OF THE NUTSHELL OF
25	QUESTIONS THAT THEN WE HAVE DELINEATED THE IMPACT
	17

1	GOALS AND THE QUESTIONS AND WE WILL DERIVE THE DATA
2	FROM.
3	LASTLY, I WANT TO PROVIDE A TIMELINE FOR
4	THIS WHOLE PROCESS. THIS IS A PRETTY ACCELERATED
5	PROCESS. WE ARE RIGHT NOW AT THE SCIENCE
6	SUBCOMMITTEE. I DON'T KNOW IF WE CAN SHOW WHERE WE
7	ARE, SARA. SO WE ARE RIGHT HERE AT THE SCIENCE
8	SUBCOMMITTEE. THE FORMATION OF THE STRATEGIC
9	ALLOCATION FRAMEWORK ANALYSIS GROUP IS AT THE
10	BOTTOM. AND AS YOU CAN SEE, WE DELINEATED THE
11	IMPACT GOALS. WE ARE DEFINING THE QUESTIONS, THE
12	VERY GRANULAR QUESTIONS, AND WE WILL START DEFINING
13	THE DATA THAT WE NEED TO ANALYZE. AND THIS IS GOING
14	TO BE BETWEEN NOW AND JUNE BY WHICH WE WILL BE
15	REPORTING TO THE STANDARDS SUBCOMMITTEE BETWEEN NOW
16	AND THE ICOC OF JUNE. OUR TEAM WILL BE REPORTING TO
17	THE SCIENCE SUBCOMMITTEE TO PROVIDE AN UPDATE ON
18	WHERE WE ARE. AND BY JUNE THERE'S GOING TO BE A
19	REVISION OF THE FLOW CONTROL EVALUATION THAT OUR
20	TEAM WILL ALSO BE PROVIDING, AND WE WILL PROVIDE A
21	FORMAL UPDATE TO THE ICOC PASSED BY THE SCIENCE
22	SUBCOMMITTEE ABOUT WHERE WE ARE IN TERMS OF THE
23	STRATEGIC ALLOCATION FRAMEWORK.
24	NOW, GIVEN THAT WE ARE DOING THIS VERY
25	STRATEGIC ALLOCATION FRAMEWORK AND PRIORITIZATION
	18

1	EXERCISE, IT DOESN'T MAKE MUCH SENSE TO PROVIDE A
2	FULL BUDGET AT THE JUNE ICOC. SO WHAT WE WOULD BE
3	PROPOSING IS TO PROVIDE AN INTERIM RESEARCH BUDGET
4	FOR THE NEXT THREE MONTHS AND A FULL OPERATIONS
5	BUDGET AT THE ICOC IN JUNE. AND THEN BY SEPTEMBER,
6	WHEN WE COME WITH A FINALIZED STRATEGIC ALLOCATION
7	FRAMEWORK RECOMMENDATIONS, WE COULD COME WITH A FULL
8	RESEARCH BUDGET ALIGNED WITH THESE RECOMMENDATIONS,
9	NOT ONLY THE RECOMMENDATIONS WILL NOT ONLY BE FOR
10	THIS YEAR OBVIOUSLY. THEY WILL BE FOR THE LIFE OF
11	CIRM.
12	AND I THINK WITH THIS, I AM ENDING MY
13	PRESENTATION. I DON'T KNOW IF YOU, J.T., WOULD LIKE
14	TO ADD ANYTHING IN TERMS OF THIS TIMELINE THAT I
15	MIGHT HAVE FORGOTTEN.
16	DR. THOMAS: NO, ROSA. I THINK THAT
17	EXPLAINS IT ALL VERY WELL. AGAIN, THIS MEETING IS
18	SPECIFICALLY ABOUT PROCESS, WHICH I THINK ROSA HAS
19	OUTLINED TO YOU. AND SO WE WELCOME ANY COMMENTS,
20	QUESTIONS, ANYTHING YOU'D LIKE, ANY INPUT ON THIS.
21	CHAIRMAN GOLDSTEIN: LET ME START THE
22	QUESTIONS WITH SOMETHING THAT PERHAPS IS A LITTLE
23	UNFAIR, BUT I AM CURIOUS ABOUT. HAVE YOU GUYS
24	DISCUSSED WHAT THE RECOMMENDATIONS MIGHT LOOK AT?
25	WILL THEY BE PROCESS ORIENTED, OR DO YOU IMAGINE

19

1	THAT YOU'RE GOING TO PICK RESEARCH FIELDS THAT LOOK
2	MORE RELEVANT OR RIPE FOR CONQUERING OR NUMBER OF
3	PATIENTS WITH PARTICULAR DISEASES? I GUESS I'M JUST
4	ASKING WHAT YOU IMAGINE THE END GAME WILL LOOK LIKE.
5	DR. THOMAS: LARRY, I THINK THE ANSWER TO
6	THAT QUESTION IS TO THIS POINT WE'VE BEEN ALL ABOUT
7	PROCESS. SO IT WOULD BE PREMATURE FOR US TO GET
8	INTO ANY DISCUSSION ON THOSE QUESTIONS, ALTHOUGH
9	THOSE ARE OBVIOUSLY VERY FAIR QUESTIONS FOR LATER
10	DOWN THE ROAD.
11	CHAIRMAN GOLDSTEIN: SURE. NO PROBLEM.
12	THANK YOU. MARIA.
13	VICE CHAIR BONNEVILLE: JUST CURIOUS.
14	WHEN THIS COMES TO THE BOARD IN SEPTEMBER, DOES THAT
15	COME ALSO WITH CONCEPT PLAN CHANGES THAT MAY BE
16	NECESSARY GIVEN PRIORITIZATION RECOMMENDATIONS THAT
17	YOU'RE MAKING? OR DOES THAT THEN MEAN THAT ONCE
18	PRIORITIZATION IS VOTED ON, THAT THEN THOSE CONCEPT
19	PLAN AMENDMENTS OR NEW CONCEPT PLANS OR NEW
20	PROGRAMS, WHATEVER MAY FLOW FROM THIS, THAT THAT'S
21	WHEN YOU START TO DEVELOP THOSE? AND THAT'S A
22	NUANCE, BUT INFORMS BUDGETARY SPEND, ETC., BETWEEN
23	JULY 1ST AND SEPTEMBER.
24	DR. THOMAS: IF I MAY ANSWER THAT ONE AS
25	WELL, MARIA. SO ANOTHER VERY GOOD QUESTION. I
	20
	20

1	BELIEVE THAT OUR OBJECT IS TO COME IN SEPTEMBER WITH
2	THE RECOMMENDATIONS HAVING HAD DISCUSSIONS ALL THE
3	WAY ALONG THE WAY TO GET TO THAT POINT SUCH THAT, AS
4	WE REACH THAT MEETING, WE WILL HAVE A PRETTY GOOD
5	SENSE OF HOW THINGS ARE GOING TO GO WITH THE BOARD.
6	AND GIVEN THAT, I WOULD PROPOSE THAT WE TO THAT
7	MEETING ALSO BRING CONCEPT PLANS BECAUSE IF WE
8	DON'T, THEN WE'RE ANOTHER THREE MONTHS BEFORE WE'RE
9	ABLE TO INTRODUCE THOSE AND IT PUSHES US THAT
10	FURTHER DOWN THE ROAD.
11	VICE CHAIR BONNEVILLE: WHICH IS EXACTLY
12	WHY I WAS ASKING. THANK YOU VERY MUCH.
13	DR. THOMAS: EXACTLY.
14	DR. HORGAN: COMMENT FROM ME IF I CAN
15	INTERJECT.
16	MS. MANDAC: NOT YET.
17	MR. TOCHER: NOT YET. WE'RE STILL GOING
18	TO THE BOARD MEMBERS. WE'LL CALL FOR PUBLIC
19	COMMENT.
20	DR. HORGAN: THANK YOU.
21	CHAIRMAN GOLDSTEIN: I GUESS I WANT TO
22	ALSO JUST MAKE A COMMENT ABOUT THE ENVIRONMENT AND
23	THEN WE'LL GET TO PAT.
24	THE PRESIDENT'S BUDGET FOR THE NIH HAS
25	ONLY A PROPOSED 1-PERCENT INCREASE IN IT, WHICH IS
	21
	21

1	MAYBE NOT SURPRISING. PRESIDENTIAL BUDGETS TEND TO
2	LOWBALL THE NIH. BUT WITH THE DYSFUNCTION IN THE
3	HOUSE AND BARELY BETTER BEHAVIOR IN THE SENATE, THE
4	RESOURCE PINCH MAY BE PARTICULARLY INTENSE FOR THE
5	NEXT COUPLE OF YEARS. I'M NOT SAYING THAT YOU GUYS
6	HAVE AN ANSWER FOR THIS, BUT IT IS SOMETHING FOR US
7	ALL TO KEEP IN MIND BECAUSE THIS IS THE CONTEXT IN
8	WHICH WE'RE GOING TO BE OPERATING.
9	PAT.
10	DR. LEVITT: I JUST WANT TO ROSA, JUST
11	WANTED CLARIFY. SO YOU HAVE THE TOTAL AVAILABLE,
12	BUT YOU HAD TWO BULLET POINTS WITH DOLLARS THAT ARE
13	SPECIFIED ALREADY. SO THE TOTAL WHICH THE PLAN IS
14	GOING TO BE ADDRESSING IS SOMETHING LIKE 2.34
15	BILLION, RIGHT? THE REMAINING BALANCE IS 3.54 MINUS
16	THESE TWO, WHICH HAVE DIRECTION IN THEM WITH
17	ALLOCATION WITH GROUPS WORKING ON HOW THESE ARE TO
18	BE DISTRIBUTED SEPARATE FROM WHAT YOU'RE TALKING
19	ABOUT. SO THE REMAINING BALANCE IS SOMETHING LIKE
20	2.34; IS THAT RIGHT?
21	DR. CANET-AVILES: THAT IS CORRECT. THAT
22	WAS IN THE DOCUMENT. VERY GOOD, PAT. YES.
23	DR. LEVITT: OKAY. I JUST WANTED
24	TO CHECK. IF YOU GO TO THE SLIDE WHICH HAS THE
25	LANDSCAPE CHANGING, DID YOUR WORK DID YOUR
	22

-	
1	MEETINGS TALK ABOUT THE DOMAINS IN WHICH THE
2	LANDSCAPE IS CHANGING MOST RAPIDLY VERSUS THOSE THAT
3	SEEM TO BE SLOWER? IS THAT PART OF YOUR ANALYSIS
4	THAT'S GOING TO OCCUR?
5	DR. CANET-AVILES: THAT IS PART OF THE
6	ANALYSIS, AND THAT COULD BE SOME OF THE
7	INFORMATION THE IDEA THAT THE LEADERSHIP TEAM
8	PRIORITIZING UNDER J.T. HAS IN TERMS OF INTERACTIONS
9	WITH THIS SCIENCE SUBCOMMITTEE COULD BE, AS WE ARE
10	DELINEATING THESE QUESTIONS THAT WILL INFORM THE
11	RECOMMENDATIONS, WE ARE GOING TO BE DELINEATING
12	TYPES OF DATA THAT WE ARE GOING TO BE ANALYZING.
13	AND WE WILL BE COMING TO YOU, NOT TO SHOW THE
14	ANALYSIS, BUT TO SHOW THE TYPES OF DATA SO THAT WHEN
15	WE COME WITH RECOMMENDATIONS AND A JUSTIFICATION AT
16	THE END, YOU CAN SAY, WELL, WHY DIDN'T YOU DO THAT?
17	BY THAT TIME YOU WILL HAVE ALREADY TOLD US. SO THAT
18	COULD BE THE KIND OF INTERACTION WE WOULD BE HAVING
19	WITH THE SCIENCE SUBCOMMITTEE, WHICH WE HOPE THAT
20	IT'S HELPFUL.
21	DR. LEVITT: OKAY. THAT SOUNDS FINE.
22	THANK YOU FOR THE CLARIFICATION.
23	CHAIRMAN GOLDSTEIN: LET ME JUST MAKE A
24	COMMENT ABOUT THIS, AND THEN WE'LL GO TO DAVID
25	HIGGINS. YOU KNOW, PAT AND OTHER MEMBERS OF THE
	23

1	NEURO TASK FORCE, I THINK AS WE START TO CLOSE IN ON
2	FINAL RECOMMENDATIONS OR MAYBE A LITTLE BIT BEFORE
3	THAT, IT WOULD CERTAINLY BEHOOVE US TO BE IN
4	CONSULTATION, ACTIVE CONSULTATION WITH THE
5	PRIORITIZATION WORK GROUP TO TRY TO MAKE SURE THAT
6	THE RECOMMENDATIONS FROM BOTH ARE HARMONIOUS. SO
7	DR. CANET-AVILES: THAT WAS WHAT THE
8	TIMELINE THIS TIMELINE, IF YOU LOOK AT THE ICOC
9	MEETING, ON THE TOP THERE ARE AN ARROW BETWEEN NOW,
10	TODAY, AND THE ICOC THAT SHOWS APRIL, MAY, JUNE TBD
11	SCIENCE SUBCOMMITTEE MEETING. SO THAT IS WHAT
12	CLAUDETTE AND SCOTT WILL HELP US SCHEDULE SO WE CAN
13	HAVE TOUCH-BASE MEETINGS THREE, FOUR BEFORE THE ICOC
14	WHERE WE WOULD BE DISCUSSING THE QUESTIONS AND THE
15	TYPE OF DATA THAT WE WILL BE COLLECTING.
16	CHAIRMAN GOLDSTEIN: GOOD.
17	DR. CANET-AVILES: THAT ALIGNS WITH WHAT
18	YOU ARE SUGGESTING AND WHAT PAT IS SUGGESTING.
19	THANK YOU.
20	DR. THOMAS: LARRY, BEFORE YOU TURN TO
21	DAVID, ROSA, COULD YOU JUST GET BACK TO LARRY'S
22	PRIOR COMMENT?
23	DR. CANET-AVILES: SO, LARRY, YOU MADE A
24	COMMENT ABOUT THE FACT THAT THE GOVERNMENT AND
25	FUNDING WILL PROBABLY BECOME HARDER TO GET. SO
	24

1	THERE WILL BE LESS FUNDING. BUT RIGHT, NOW EVEN
2	WITHOUT THAT PREDICAMENT, OUR GATES ARE FLOODED. SO
3	WE HAVE NO IDENTITY IN SOME WAYS AS CIRM. AND ONE
4	OF THE THINGS THAT WE ARE TRYING TO DO WITH THIS
5	IMPACT GOAL DEFINITION AND STRATEGIC ALLOCATION
6	FRAMEWORK IS TO DEFINE WHO ARE WE AND HOW ARE WE
7	GOING TO GET THERE AND MAKE SURE THAT WE GET THERE
8	IN THE YEARS THAT WE HAVE WITH THE FUNDING INSTEAD
9	OF LIKE JUST KEEP FUNDING AND NOT GETTING TO A CURE
10	OR TO THESE DEFINED IMPACT GOALS. THAT'S THE
11	DIFFERENCE.
12	DR. THOMAS: I THINK, LARRY, WITH RESPECT
13	TO YOUR COMMENT ABOUT THE NIH BUDGET, IT JUST
14	UNDERSCORES THE IMPORTANCE OF THIS PROCESS THAT
15	WE'RE UNDERTAKING RIGHT HERE.
16	CHAIRMAN GOLDSTEIN: ABSOLUTELY, YES.
17	DR. THOMAS: WE'RE VERY AWARE OF THAT AND
18	ARE PROCEEDING ACCORDINGLY.
19	CHAIRMAN GOLDSTEIN: YES. IT'S GOING TO
20	BE CHAOS IN WASHINGTON. DAVID HIGGINS.
21	DR. HIGGINS: JUST A SMALL, PROBABLY
22	TRIVIAL QUESTION, BUT YOU TALKED ABOUT PROCESS AND
23	HOW THAT MIGHT CHANGE, AND WE TALKED ABOUT THE MONEY
24	THAT WE HAVE TO SPEND TO EFFECT THAT CHANGE. HOW
25	FAR DOWN DO YOU SEE THE GROUP PARSING THE CHOICES?
	25

1	ARE YOU GOING TO CHOOSE BY INDICATION, BY JUST THE
2	APPLICATIONS THAT COME IN OVER THE TRANSOM, OR IS
3	THERE SOME OTHER SLICE AND DICE MECHANISM THAT WE
4	HAVEN'T USED BEFORE THAT WE'RE GOING TO USE THAT
5	FAVORS A CERTAIN INDICATION OR CERTAIN CLASS?
6	DR. THOMAS: DAVID, THE ANSWER IS, AGAIN,
7	WE HAVEN'T GOTTEN TO THAT LEVEL OF DISCUSSION YET.
8	WE'RE STILL ON THE PROCESS, BUT ALL OF THAT WILL BE
9	A PART OF IT FOR SURE. AND I THINK EVERYTHING IS ON
10	THE TABLE IS THE ANSWER TO YOUR QUESTION. WE WILL
11	LOOK AT ALL OPTIONS, WEIGH ALL OPTIONS, AND MAKE
12	RECOMMENDATIONS ACCORDINGLY.
13	DR. CANET-AVILES: AND THE KEY WILL BE THE
14	DATA. THE DATA WILL JUSTIFY, AND YOU WILL BE
15	EMBRACING THE PROCESS AND THE DATA.
16	DR. THOMAS: CORRECT.
17	DR. HIGGINS: I CAN IMAGINE, AND THE
18	REASON FOR MY QUESTION IS I CAN IMAGINE HAVING A
19	MIXTURE OF PROJECTS OR PRODUCTS, BUT BASED, NOT ON
20	SOMEBODY'S FAVORING THAT, BUT MORE WHAT'S THE
21	EQUILIBRIUM FOR THE MONEY SPENT TO GET A SORT OF
22	POOL OF PROJECTS THAT MAKE SENSE. THAT WASN'T A
23	QUESTION. THAT WAS JUST ME RANTING.
24	CHAIRMAN GOLDSTEIN: THERE'S A HAND UP IN
25	THE CONFERENCE ROOM. I DON'T KNOW WHO IS TRYING TO
	26

1	SPEAK THERE.
2	DR. MELMED: I AM.
3	MS. MANDAC: VITO.
4	CHAIRMAN IMBASCIANI: I'LL WAIT TILL
5	AFTER. SHLOMO, GO AHEAD.
6	DR. MELMED: I THINK IT'S VERY WELL
7	THOUGHT THROUGH. THANK YOU. MY CONCERN IS MORE ON
8	THE NONSCIENTIFIC SIDE. I'M VERY CONCERNED WITH
9	MESSAGING. AND WE DON'T WANT THIS TO TURN INTO A
10	NEGATIVE OPPORTUNITY TO BASH CIRM. AND SO I WOULD
11	LIKE TO SEE FROM STAFF A PLAN FOR MESSAGING WITH
12	ACTUAL BULLET POINTS BECAUSE OTHERWISE WE'RE GOING
13	TO BE SWAMPED WITH NEGATIVE PRESS. I CAN JUST SEE
14	WHAT THE BLOGGERS ARE GOING TO WRITE. I THINK IT
15	STARTED ALREADY WHEN WE FIRST RAISED IT A COUPLE OF
16	MEETINGS AGO. THERE WERE SOME NEGATIVE COMMENTS
17	ONLINE. AND SO I'M JUST RAISING THAT FOR STAFF TO
18	CONSIDER FOR US.
19	DR. THOMAS: THANK YOU. WE WILL
20	DEFINITELY TAKE THAT ADVICE. THANK YOU, SHLOMO.
21	CHAIRMAN GOLDSTEIN: BACK TO THE
22	CONFERENCE ROOM THEN.
23	CHAIRMAN IMBASCIANI: THANKS, LARRY. THIS
24	IS VITO.
25	SO THIS IS A QUESTION IN THE FORM OF A
	27

1	COMMENT OR A COMMENT IN THE FORM OF A QUESTION. THE
2	FINANCE COMMITTEE I'LL MAKE SURE PRESENTS A BUDGET
3	TO THE BOARD ON TIME. AND YOU KNOW OUR BUDGETS
4	WHICH COVER ADMINISTRATIVE AND RESEARCH COSTS ARE
5	BASED ON CURRENT EXPERIENCE AND A LOOK BACK AT
6	HISTORY PLUS A LITTLE COLA, IF YOU WILL, FUDGE
7	FACTOR. AND THAT'S GOING TO BECOME OUR BUDGET, OUR
8	FUNDING DOCUMENT STARTING JULY 1. BUT AS WE SEE IN
9	THIS PRESENTATION, THERE'S GOING TO BE AN ASK OF THE
10	BOARD, WE KNOW IT NOW, IN SEPTEMBER FOR A REVISION.
11	AND SO I'M ALREADY STARTING TO WONDER, WELL, THIS IS
12	A DELTA TO OUR BUDGET. IS IT GOING TO BE A PLUS OR
13	IS IT GOING TO BE A NEGATIVE OR IS IT GOING TO BE IN
14	THE BALLPARK? THE SIZE OF THE DELTA IS WHAT
15	CONCERNS ME ALREADY.
16	DR. CANET-AVILES: SO THANK YOU FOR THE
17	QUESTION, DR. IMBASCIANI. SO AS WE MENTIONED, FOR
18	THE OPERATIONS, THE BUDGET WILL BE THE SAME. THERE
19	WILL BE NO CHANGE. BUT AT THE JUNE MEETING, WE WILL
20	BE PRESENTING AN INTERIM BUDGET. WE ALREADY KNOW
21	WHICH PROGRAMS ARE SLOTTED. THERE ARE THREE
22	PROGRAMS THAT ARE SLOTTED BETWEEN JUNE AND
23	SEPTEMBER, AND WE ARE GOING TO BUDGET FOR THOSE
24	PROGRAMS. THEY ARE IN TWO OF OUR IN DR.
25	CREASEY'S AND MY TEAM.

28

1	AND THEN AT THE SEPTEMBER MEETING, GIVEN
2	WHAT WE WERE JUST DISCUSSING IN TERMS OF
3	PRIORITIZATION AND NEW CONCEPTS AND MODIFIED
4	CONCEPTS, THAT'S GOING TO BE A DELTA THAT'S GOING TO
5	BE POSITIVE IN THE SENSE THAT WE WILL BE ASKING FOR
6	MOST OF THE MONEY FOR THAT YEAR'S ALLOCATION. SO
7	THE DELTA WILL BE POSITIVE. DOES THAT MAKE SENSE?
8	CHAIRMAN IMBASCIANI: IT DOES. I GUESS
9	IT'S THE SIZE OF THE DELTA.
10	DR. CANET-AVILES: THE SIZE OF DELTA WILL
11	BE AROUND THE NORMAL ALLOCATION MOST LIKELY THAT WE
12	MAKE, BUT I CAN'T REALLY MAKE A PREDICTION BECAUSE
13	THE RECOMMENDATIONS ARE GOING TO GUIDE THAT. BUT I
14	COULD SAY THAT IT'S GOING TO BE ABOUT THE NORMAL
15	BUDGET THAT WE HAVE FOR A YEAR, BUT WE CAN'T
16	PREDICT. IT WILL BE ALIGNED WITH THE
17	RECOMMENDATIONS.
18	DR. THOMAS: CORRECT.
19	CHAIRMAN IMBASCIANI: WONDERFUL. THANK
20	YOU.
21	CHAIRMAN GOLDSTEIN: OTHER QUESTIONS OR
22	COMMENTS?
23	SO LET ME JUST REITERATE WHAT SHLOMO SAID.
24	I THINK IT'S A VERY GOOD IDEA TO GET THE
25	COMMUNICATIONS TEAM AND THE COMMUNICATIONS
	29

1	SUBCOMMITTEE WORKING ON MESSAGING FOR THE SET OF
2	ISSUES THAT ARE SURELY COMING. AND MAYBE WE CAN GET
3	AHEAD OF IT, WHICH WOULD BE TERRIFIC. AND SINCE THE
4	COMMUNICATIONS SUBCOMMITTEE HAS SOMETIMES BEEN
5	SEARCHING A LITTLE BIT FOR A MISSION, THIS COULD BE
6	A VERY GOOD THING TO WORK WITH. AS RECOMMENDATIONS
7	ARE STARTING TO BE ROLLED OUT, PERHAPS WE CAN BE
8	AHEAD OF THEM.
9	KEITH, IS THERE ANYTHING FROM YOUR
10	EXPERIENCE IN WASHINGTON THAT MIGHT INFORM HOW WE GO
11	ABOUT THIS WITHOUT GETTING IN TROUBLE WITH POWERFUL
12	INTEREST GROUPS AROUND THE STATE?
13	DR. YAMAMOTO: I DON'T KNOW. I'M NOT SURE
14	WHAT KIND OF TROUBLE YOU'RE TALKING ABOUT, LARRY.
15	BUT I THINK THAT THE COMMENT THAT YOU MADE EARLIER
16	ABOUT THE STATE OF THE BUDGET IS GOING TO MAKE WHAT
17	CIRM IS DOING MORE VISIBLE THAN EVER AND A BIGGER
18	TARGET THAN EVER. SO I THINK THAT GETTING THIS
19	RIGHT NOW, GETTING OUR PROCESS CORRECT NOW, REALLY
20	FOCUSED ON PROCESS IS GOING TO BE AN ESSENTIAL
21	ELEMENT OF NOT GETTING IN TROUBLE. BUT I'M NOT
22	SEEING ANYTHING RIGHT NOW AT THE NATIONAL LEVEL THAT
23	IS SOMETHING THAT WE HAVE TO PAY PARTICULAR NOTE TO
24	EXCEPT FOR THE POINT THAT YOU MADE ABOUT THE BUDGET.
25	CHAIRMAN GOLDSTEIN: GREAT. THANKS.
	30

30

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1	SORRY TO PUT YOU ON THE SPOT, BUT I COULDN'T RESIST.
2	OTHER COMMENTS OR QUESTIONS FROM THE
3	GROUP? IF NOT, SCOTT REMINDS ME THAT IT'S TIME TO
4	OPEN IT UP FOR PUBLIC COMMENT THEN.
5	MR. TOCHER: THAT'S RIGHT, LARRY. I
6	BELIEVE RICHARD HORGAN HAD A COMMENT. RICHARD, ARE
7	YOU ON THE LINE?
8	DR. HORGAN: YEAH. CAN YOU ALL HEAR ME
9	ΟΚΑΥ?
10	MR. TOCHER: JUST A REMINDER THAT YOU HAVE
11	THREE MINUTES FOR PUBLIC COMMENT. SO PLEASE
12	PROCEED.
13	DR. HORGAN: THREE OR LESS, YOU GOT IT.
14	SO THANK YOU FOR TAKING THE TIME TO LISTEN TO ME.
15	MY NAME IS RICHARD HORGAN, AND I'M THE FOUNDER AND
16	CEO OF A NON-PROFIT BIOTECH CALLED CURE RARE
17	DISEASE. WE WORK TO DEVELOP ULTRA RARE THERAPEUTICS
18	FOR NEGLECTED AND OVERLOOKED POPULATIONS ACROSS THE
19	UNITED STATES PRIMARILY, BUT ALSO CANADA AND WESTERN
20	EUROPE.
21	WE DO THIS THROUGH A COLLABORATION OF
22	ACADEMICS, SOME OF WHOM ARE IN THE VERY STATE THAT
23	CIRM IS LOCATED IN.
24	SO A COUPLE POINTS TO MAKE. SO I COME AT
25	THIS REPRESENTING THE ULTRA RARE DISEASE COMMUNITY.
	31

1	ONE OF THE COMMENTS MADE, I THINK, BY GENTLEMAN
2	SHLOMO'S POINT ABOUT MESSAGING, I STRONGLY AGREE
3	THAT THIS WILL LOOK VERY POOR FROM AN OPTICS
4	PERSPECTIVE FROM THE ULTRA RARE DISEASE COMMUNITY,
5	MANY OF WHOM HAVE ALREADY REACHED OUT VERY
6	FRUSTRATED THAT ONE OF THE ONLY MECHANISMS AVAILABLE
7	TO ADVANCE PROMISING SCIENCE INTO THE CLINIC HAS NOW
8	BEEN PAUSED.
9	FOR CONTEXT, ULTRA RARE DISEASE PATIENTS,
10	ALTHOUGH THERE'S NO OFFICIAL DEFINITION, IS
11	INFORMALLY NONCOMMERCIAL DISEASES THAT ARE IMPACTING
12	POPULATIONS THAT ARE SORELY NEGLECTED AND
13	OVERLOOKED.
14	SO WHAT I REALLY, REALLY, REALLY ENCOURAGE
15	YOU ALL TO CONSIDER AS PART OF THIS PROCESS, AND I
16	UNDERSTAND YOU'RE IN THE PROCESS OF DEVELOPING PART
17	OF THIS, IS EQUITY AND WHO RECEIVES IT. MANY OF THE
18	DISEASES THAT WE WORK WITH, THOSE THAT ARE
19	NONCOMMERCIAL, THOSE THAT WE CALL ULTRA RARE HAVE NO
20	OTHER OPPORTUNITY TO RECEIVE ANY SORT OF FUNDING TO
21	ADVANCE PROGRAMS BEYOND THAT PRE-IND STAGE.
22	FAMILIES, MANY OF WHOM I'VE ENCOURAGED TO
23	CALL TO JOIN THE NEXT CALL LATER THIS WEEK, HAVE
24	WORKED VERY HARD AND VERY TIRELESSLY TO RAISE MONEY
25	TO WORK WITH ACADEMIC SCIENTISTS TO A DEVELOP A
	22

1	THERAPEUTIC TO A CERTAIN STAGE. HOWEVER, ONCE YOU
2	GET TO THAT PRE-IND STAGE, SCALING UP MANUFACTURING
3	AND GLP TOXICOLOGY BECOMES A VERY, VERY EXPENSIVE
4	EFFORT WHICH VERY FEW FAMILIES CAN SUPPORT.
5	AND SO I REALLY STRONGLY CONSIDERED AND
6	ENCOURAGED THIS GROUP TO CONSIDER THE ULTRA RARE
7	DISEASE COMMUNITY THAT HAS NO OTHER AVAILABLE ROUTE
8	TO FINANCING FOR THE DEVELOPMENT. OTHER DISEASES
9	THAT PARTICIPATE IN THIS PROGRAM SUCH THAT ARE
10	COMMERCIALIZABLE DO HAVE OTHER OPTIONS TO RAISE
11	FUNDING, VENTURE FUNDING AND OTHER GRANT FUNDING
12	THAT HISTORICALLY THESE VEHICLES HAVE SUITED THESE
13	POPULATIONS BETTER. BUT AGAIN, THE ULTRA RISK
14	DISEASE COMMUNITY DOES NOT HAVE ACCESS TO THESE
15	OPTIONS.
16	EXISTING APPLICATIONS FOR CLINICAL
17	DEVELOPMENT PROGRAMS ONE AND TWO I WOULD NOTE HAVE
18	BEEN VETTED BY THE FDA. AND WITH THESE PROGRAMS
19	THAT WE WORK WITH ESPECIALLY FUNDING IS THE MAJOR
20	ROADBLOCK HERE TO TREATING THESE PATIENTS. AND SO I
21	CANNOT EMPHASIZE ENOUGH THE DIRE STRAIGHT THAT THIS
22	PATIENT POPULATION IS IN BEING OVERLOOKED, BEING
23	NEGLECTED, AND HAVING NO ACCESS TO CAPITAL TO ENGAGE
24	IN CAPITAL INTENSIVE EXERCISES SUCH AS GMP
25	MANUFACTURING, SUCH AS GLP TOXICOLOGY STUDIES TO GET

1	A DRUG INTO THE CLINIC. SO PLEASE CONSIDER THIS
2	GROUP AS A VERY VULNERABLE, VERY SENSITIVE GROUP
3	THAT WILL PAY A VERY, VERY, VERY STEEP PRICE FOR NOT
4	HAVING ACCESS TO FUNDING THAT THIS INCREDIBLE
5	PROGRAM THAT YOU ALL CHAMPION ALLOWS THEM TO HAVE.
6	THE WRONG DECISION HERE, DON'T GET ME
7	WRONG, WILL COST LIVES. THESE ARE FAMILIES THAT ARE
8	DESPERATE TO BE ABLE TO ADVANCE THERAPEUTICS AND
9	WORK WITH GREAT ACADEMICS, SOME OF WHOM ARE ON THIS
10	CALL, TO TRY TO TAKE A SHOT AT HELPING THOSE
11	HELPING THEIR LOVED ONES NOT DIE FROM THESE
12	DISEASES.
13	AND SO IN CLOSING, PLEASE CONSIDER THE
14	ULTRA RARE DISEASE POPULATION. PLEASE CONSIDER THE
15	EQUITY OF THE PROCESS THAT YOU ARE DEVELOPING IN
16	RELATION TO THESE NEGLECTED AND OVERLOOKED DISEASES.
17	PLEASE CONSIDER THAT PROGRAMS HAVING APPLICATIONS
18	SUBMITTED FOR CLIN1 AND 2 HAVE BEEN VETTED BY THE
19	FDA VIA A PRE-IND MEETING, AND PLEASE DON'T LEAVE
20	THESE PATIENTS BEHIND BECAUSE ALMOST EVERY OTHER
21	OPPORTUNITY HAS LEFT THESE PATIENTS BEHIND FROM
22	VENTURE CAPITAL TO THE VAST MAJORITY OF NIH
23	PROGRAMS. CIRM IS ONE OF THE ONLY BEACONS OF HOPE
24	THAT THESE ULTRA RARE DISEASE PATIENTS HAVE, AND
25	PLEASE DON'T LET THEM DOWN. THANK YOU VERY MUCH FOR

1	THE OPPORTUNITY TO MAKE COMMENT. IF THERE'S
2	ANYTHING ELSE I CAN DO, PLEASE LET ME KNOW.
3	CHAIRMAN GOLDSTEIN: THANK YOU,
4	DR. HORGAN.
5	MR. TOCHER: I DON'T SEE ANY OTHER HANDS
6	RAISED. NO. I THINK THAT WILL COVER THE PUBLIC
7	COMMENT FOR THE MEETING.
8	CHAIRMAN GOLDSTEIN: GREAT. I THINK WE
9	DON'T HAVE ANY ADDITIONAL AGENDA ITEMS, BUT WHAT
10	ROSA AND THE OTHERS HAVE KOREN TEMPLE-PERRY
11	PLEASE.
12	MS. TEMPLE-PERRY: HI, ALL. I JUST WANTED
13	TO DROP IN AND JUST MENTION REGARDING MESSAGING THAT
14	THE COMMUNICATIONS TEAM WILL START WORKING ON THIS.
15	I'LL WORK WITH THE COMMUNICATIONS SUBCOMMITTEE TO
16	START WORKING THROUGH MESSAGING AND ANY SORT OF
17	OTHER NEEDS TO COMMUNICATE THAT. SO WE DID HEAR
18	THAT LOUD AND CLEAR. I JUST WANTED TO POP ON AND
19	JUST LET YOU KNOW THAT THAT'S SOMETHING THAT WE WILL
20	PRIORITIZE.
21	CHAIRMAN GOLDSTEIN: GREAT. THANK YOU
22	VERY MUCH, KOREN. APPRECIATE IT. ANYTHING ELSE
23	ANYBODY WANTS TO ADD? I DON'T SEE ANY HANDS. THANK
24	YOU, MARIA. THUMB IS UP, NOT A HAND. SHE'S
25	EXPRESSING APPROVAL ON A SILENT WAY. OKAY. WITH
	35

1	THAT, I SUGGEST THAT WE ADJOURN. THERE'S A LOT OF
2	WORK TO BE DONE. THANK YOU ALL.
3	MR. TOCHER: THANK YOU, LARRY.
4	(THE MEETING WAS THEN CONCLUDED AT 3:45 P.M.)
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