

**BETH C. DRAIN, CA CSR NO. 7152**

BEFORE THE  
SCIENCE SUBCOMMITTEE AND 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, TREATMENTS, AND CURES  
INITIATIVE OF 2020  
REGULAR MEETING

LOCATION: VIA ZOOM

DATE: JUNE 14, 2014  
12 P.M.

REPORTER: BETH C. DRAIN, CA CSR  
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MS. MANDAC: MARK, WE DO HAVE QUORUM SO WE CAN START.

MR. TOCHER: MARK, I THINK YOU'RE ON MUTE, OR AT LEAST WE'RE HAVING TROUBLE HEARING YOU HERE IN HEADQUARTERS.

MR. FISCHER-COLBRIE: SORRY ABOUT THAT NATURALLY. VITO, IF YOU WANT TO GO AHEAD.

CHAIRMAN IMBASCIANI: YES. ALL RIGHT, EVERYONE, AND THANK YOU FOR JOINING. THIS IS A JOINT MEETING OF THE NEURO TASK FORCE AND THE SCIENCE SUBCOMMITTEE, CO-CHAired BY MARK FISCHER-COLBRIE. THE OTHER CO-CHAIR, LARRY GOLDSTEIN, HAS RESIGNED FROM THE BOARD, AS YOU KNOW. PLEASE REMEMBER THAT, UNLESS I'M MISTAKEN, THAT MARK FISCHER-COLBRIE IS ALSO CO-CHAIR OF THE NEURO TASK FORCE.

MR. TOCHER: NO, THAT'S NOT CORRECT. JUST LARRY WAS THE CHAIR. SO WE HAVE A VACANCY IN THE CHAIR FOR THE TASK FORCE. AND I THINK AT THIS POINT, JUST FOR PROTOCOL, IT WOULD BE HELPFUL, IF THERE IS NO OBJECTION, FOR THE PURPOSES JUST OF THIS ONE MEETING UNTIL THE FULL BOARD MEETS AND CAN SELECT A NEW CHAIR ON THE 27TH, IF THERE IS NO

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OBJECTION, SINCE MARK CHAIRS THE SCIENCE SUBCOMMITTEE, IF MARK COULD ALSO CHAIR THIS MEETING OF THE NEURO TASK FORCE, THEN THAT WOULD PROBABLY SIMPLIFY OUR MEETING TODAY.

CHAIRMAN IMBASCIANI: THAT IS MY REQUEST IF THERE'S NO OBJECTION.

DR. YAMAMOTO: NO OBJECTION.

MR. TOCHER: GREAT. THANKS VERY MUCH.

CHAIRMAN IMBASCIANI: SCOTT, I THINK YOU MAY, SINCE WE'VE BEEN CALLED TO ORDER, YOU CAN CALL THE ROLL IF YOU'RE DOING THAT.

MR. TOCHER: SURE. NOW THE MEETING, I THINK, IS CHAIRED BY MARK. SO I ANTICIPATE MARK WOULD DO THE SAME THING. SO LET'S START WITH THE ROLL.

CHAIRMAN FISCHER-COLBRIE: YEAH, THANK YOU. YOU'RE GOING TO HAVE TO TRAIN ME. SCOTT, IF YOU COULD CALL THE ROLL, THAT WOULD BE TERRIFIC. THANK YOU.

MS. MANDAC: I'LL DO IT FOR TODAY. MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: PRESENT.

MS. MANDAC: LEONDRAL CLARK-HARVEY.  
DEBORAH DEAS. MARK FISCHER-COLBRIE.

CHAIRMAN FISCHER-COLBRIE: YES, HERE.

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MS. MANDAC: FRED FISHER.

DR. FISHER: PRESENT.

MS. MANDAC: ELENA FLOWERS. JUDY GASSON.

DR. GASSON: HERE.

MS. MANDAC: DAVID HIGGINS.

DR. HIGGINS: PRESENT.

MS. MANDAC: VITO IMBASCIANI.

CHAIRMAN IMBASCIANI: PRESENT.

MS. MANDAC: STEVE JUELSGAARD. PAT

LEVITT.

DR. LEVITT: PRESENT.

MS. MANDAC: SHLOMO MELMED.

DR. MELMED: PRESENT.

MS. MANDAC: LAUREN MILLER-ROGEN. CHRIS

MIASKOWSKI. MARV SOUTHARD.

DR. SOUTHARD: PRESENT.

MS. MANDAC: KAROL WATSON. KEITH

YAMAMOTO.

DR. YAMAMOTO: HERE.

MS. MANDAC: WE HAVE QUORUM FOR BOTH  
SUBCOMMITTEES. BACK TO YOU, MARK.

CHAIRMAN FISCHER-COLBRIE: SO WE'VE GOT A  
LOT TO COVER, BUT JUST TO GIVE ADDITIONAL BACKGROUND  
OR CONTEXT, THEN I'LL LET OTHERS WEIGH IN ON THIS AS  
WELL. WE ARE IN A SITUATION WHERE WE HAVE A LOT OF

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ACTIVITIES MOVING FORWARD, THE STRATEGIC ALLOCATION FRAMEWORK THAT WE'LL HAVE FURTHER DISCUSSION, THE GREAT WORK ON THE NEURO TASK FORCE, AND THE OVERLAY WITH THE SCIENCE COMMITTEE ON BOTH OF THOSE PROCESSES, IF YOU WILL. AND WE UNFORTUNATELY HAVE INABILITY TO DRAW UPON LARRY'S TREMENDOUS TALENTS. SO I HAVE GREAT CONFIDENCE THAT THE TEAM WILL CONTINUE TO AID ME IN THE VARIOUS ACTIVITIES HERE GOING FORWARD.

SO WITH THAT IN MIND, THIS IS THEN A JOINT MEETING OF THE NEURO TASK FORCE AND THE SCIENCE SUBCOMMITTEE. AND FROM THAT PERSPECTIVE, IT'S IMPORTANT TO PROVIDE AN UPDATE ON WHERE THINGS ARE HEADED, WHERE THINGS ARE GOING, AND TO BE ABLE TO HAVE IMPORTANT DISCUSSIONS ABOUT OUR CONSIDERATIONS FOR NEXT STEPS.

AND WITH THAT, THE CIRM STAFF HAS BEEN DOING A BEYOND-THE-PALE JOB AND EFFORT AROUND HARNESSING THESE VARIOUS INITIATIVES INTO A COHESIVE WHOLE AND A COHESIVE CONTEXT. AND I HAVE TO APPLAUD THEM BECAUSE THEY'RE SPENDING A HUGE AMOUNT OF TIME, EXTREMELY LARGE AMOUNT OF PHYSICAL AND PSYCHIC ENERGY. AND FROM THE ACTIVITIES THAT I'VE SEEN TODAY, I'M EXTREMELY IMPRESSED AT WHERE THE TEAM HAS ADVANCED.

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AND IN THE CHARACTERIZATION OF THAT ACTIVITY, IT'S IMPORTANT TO NOTE THAT, BECAUSE THINGS HAVE BEEN MOVING FAIRLY QUICKLY, THE FULL INTENTION IS TO ENSURE THE DIRECT INVOLVEMENT OF ALL THE PEOPLE ON THIS CALL, THE DIRECT INVOLVEMENT OF ALL THE FOLKS ON THE BOARD IN THOSE OTHER ACTIVITIES. SO DON'T WANT THERE TO BE A PRESUMPTION THAT THE TEAM IS OFF AND RUNNING AND GRUMBLING IN A CORNER IN COMING UP WITH ANSWERS TO A LOT OF THESE THINGS ON THEIR OWN; BUT, IN FACT, ARE INCREDIBLY DILIGENT AT MOVING THE BALL FORWARD. AND IT'S IMPORTANT, THEN, TO HAVE THE FRAMING FOR THIS GROUP AND OTHERS AS TO HOW TO HAVE THE BEST CONVERSATIONS GOING FORWARD.

SO JUST A LITTLE BIT OF A PREAMBLE. VITO, I DON'T KNOW IF THERE'S ANYTHING ELSE THAT YOU WOULD LIKE TO ADD TO THAT.

CHAIRMAN IMBASCIANI: NO, NOT AT THIS POINT.

CHAIRMAN FISCHER-COLBRIE: OKAY. WITH THAT, I'LL TURN IT OVER TO THE CIRM STAFF TO PROVIDE THE CURRENT CONTEXTUAL UPDATES FOR WHERE WE'RE AT AND WHAT INTENTIONS ARE GOING FORWARD.

DR. THOMAS: THANKS VERY MUCH, MARK. I HAD A FEW OPENING REMARKS TO MAKE HERE. BUT YOU

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PHRASED THINGS SO ELOQUENTLY AND COMPREHENSIVELY THAT IT REALLY PREEMPTED MY COMMENTS. SO THANK YOU VERY MUCH. OTHER THAN TO POINT OUT THAT THIS HAS BEEN A PROCESS THAT ROSA AND SARA HAVE LED WITH A VERY CONSIDERABLE AMOUNT OF TIME DEVOTED BY MEMBERS OF THE LEADERSHIP TEAM, AS MARK SAID, IN CONSULTATION WITH ALL OF YOU AND THOSE OF YOUR COLLEAGUES WHO WEREN'T ABLE TO MAKE THIS CALL WHICH HAS LED TO THE UPDATE THAT ROSA IS GOING TO GIVE YOU TODAY.

JUST TO REMIND, THIS IS PART AND PARCEL OF A MAJOR, HEAVY LIFT BEING UNDERTAKEN BY THE CIRM FAMILY WRIT LARGE HERE THIS YEAR, OF WHICH THIS PARTICULAR PART OF THE EFFORT IS SORT OF THE ONE THAT FACTORS IN ALL THE DIFFERENT ELEMENTS THAT ARE PART OF THAT TEAMWORK.

SO I THINK THIS IS A GOOD CHECK FOR MEMBERS OF THE SCIENCE SUBCOMMITTEE AND THE NEURO TASK FORCE. AND I WOULD REMISS IF I JUST DIDN'T GIVE A BIT OF FURTHER COMMENT ON LARRY, WHO WE'RE VERY SORRY TO SEE RESIGN FROM THE BOARD, WHO HAS BEEN A HUGE FORCE IN THE CIRM EFFORT FOR MANY, MANY YEARS GOING ALL THE WAY BACK TO HIS SUPPORT FOR PROP 71 WHEN HE WAS A RESEARCH SCIENTIST AT UCSD AND THROUGH HIS TIME WAS HEAD OF THE STEM CELL PROGRAM,



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HE WAS A GRANTEE, HE KNEW ALL ABOUT WHAT CIRM DID BEFORE HE JOINED THE BOARD AS THE DESIGNATE FOR UCSD, AND HAS BEEN A TREMENDOUS ASSET, NOT JUST FROM HIS OWN PERSONAL KNOWLEDGE, BUT FROM HIS NETWORK, THE RESPECT HE'S HELD THROUGHOUT THE NEURO WORLD THROUGHOUT ALL OF HIS TIME IN THE SPACE. AND WE HAVE BENEFITED TREMENDOUSLY BY HIS LEADERSHIP BOTH WITH THE NEURO TASK FORCE AND THE SCIENCE SUBCOMMITTEE AND THE ICOC AT LARGE.

SO, LARRY, I'M SURE YOU'RE PROBABLY WATCHING. TIP OF THE CAP TO YOU. AND WE'LL BE ABLE TO GIVE YOU A MORE THOROUGH APPRECIATION DOWN THE ROAD, BUT DIDN'T WANT THIS MEETING TO PASS WITHOUT MENTIONING YOUR MASSIVE CONTRIBUTION, OF WHICH THIS IS A PRODUCT. SO WITH THAT, I'LL TURN THINGS OVER TO ROSA TO PRESENT.

CHAIRMAN FISCHER-COLBRIE: J.T., IF I CAN ADD ONE MORE COMMENT TO THIS. IN ADDITION TO THE HERCULEAN EFFORT THAT STAFF HAS DONE, I'VE BEEN EXTREMELY IMPRESSED AT THE CROSS-POLLENIZATION ACROSS ALL THE TEAM MEMBERS, THE COHESIVE NATURE OF THIS. AND I ALSO WOULD BE REMISS IN THIS CONTEXT OF NOT ACKNOWLEDGING YOUR TREMENDOUS LEADERSHIP ON THIS ACTIVITY AS PART OF THAT EFFORT. SO THANK YOU FOR THOSE INTRODUCTORY COMMENTS.

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ROSA, IF YOU WANT TO TAKE IT AWAY, THAT'S GREAT.

DR. CANET-AVILES: THANK YOU.

DR. THOMAS: THANKS, MARK.

DR. CANET-AVILES: THANK YOU, J.T. THANK YOU, MARK, AND THANK YOU, MEMBERS OF THE SCIENCE SUBCOMMITTEE. AND I WANT TO ECHO J.T.'S COMMENTS WITH REGARDS DR. GOLDSTEIN. HIS SCIENTIFIC POWER AND WEIGHT IS BEING MISSED. WE MISS HIM VERY MUCH IN THE DEVELOPMENT OF THIS NEURO TASK FORCE AND SCIENCE SUBCOMMITTEE.

SO WITH THAT SAID, THE PRESENTATION TODAY, THIS SLIDE PROVIDES AN OVERVIEW OF HOW WE HAVE STRUCTURED TODAY'S UPDATE. AND YOU WILL BE ABLE TO TRACK WHICH SECTIONS WE ARE IN BY -- YOU WILL BE ABLE TO TRACK WHICH SECTIONS WE ARE DISCUSSING IF YOU LOOK AT THE TOP RIGHT OF THE PRESENTATION, THE TOP RIGHT CORNER OF EACH SLIDE.

SO WITH THAT SAID, AS A QUICK BACKGROUND CONTEXT FOR THE STRATEGIC ALLOCATION FRAMEWORK, THIS SLIDE PRESENTS AN OVERVIEW OF CIRM'S FIVE-YEAR STRATEGIC PLAN THAT STARTED IN 2022 AND THAT IS ORGANIZED IN THREE MAIN THEMES THAT WE ARE ALL VERY FAMILIAR WITH.

FOR CONTEXT, THE STRATEGIC ALLOCATION

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FRAMEWORK WE ARE PRESENTING DETAILS IMPACT GOALS, SUCCESS METRICS, AND RECOMMENDATIONS FOR THE ONGOING IMPLEMENTATION OF THE CIRM STRATEGIC PLAN.

SO OVER THE PAST 17 YEARS, I KNOW WE ARE ON OUR 20TH ANNIVERSARY, BUT WE WERE NOT ABLE TO START RELEASING PROGRAMS TILL 2007-8. SO IT'S OVER THE PAST 17 YEARS THAT CIRM HAS BEEN AT THE FOREFRONT OF THE STEM CELL AND REGENERATIVE MEDICINE FIELD. OUR INSTITUTE HAS BEEN INSTRUMENTAL IN FUNDING CUTTING-EDGE RESEARCH, DEVELOPING ROBUST INFRASTRUCTURE, ALSO PIONEERING EDUCATIONAL PROGRAMS FROM HIGH SCHOOL TO M.D./PH.D. SCHOLARS AND UNDERSERVED COMMUNITIES, AND ALSO CATALYZING THE PROGRESSION FROM REGENERATIVE MEDICINE RESEARCH TO PRACTICAL APPLICATIONS.

THIS DIVERSE INVESTMENT HAS ALLOWED US TO PUSH THE BOUNDARIES OF WHAT'S POSSIBLE IN REGENERATIVE MEDICINE NOW. SINCE OUR INCEPTION, THERE ALSO BEEN SIGNIFICANT GROWTH IN THE REGENERATIVE MEDICINE FIELD. AND THIS RAPID EXPANSION HAS BEEN MIRRORED BY AN INCREASING DEMAND FOR OUR RESOURCES, WHICH, AS WE ARE ALL IN THE KNOW, THEY ARE FINITE.

SO THE DEMAND FOR FUNDING NOW SIGNIFICANTLY SURPASSES WHAT WE ARE ABLE TO PROVIDE.

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WE HAVE HAD A TASTE OF THAT WITH THE CLINICAL APPLICATIONS AND THE CIRM CLINICAL CONTROL FLOW THAT WE HAD TO IMPLEMENT. SO GIVEN THIS SCENARIO, IT IS IMPERATIVE THAT WE CAREFULLY CONSIDER HOW TO ALLOCATE OUR REMAINING RESOURCES.

THE FRAMEWORK THAT HAS BEEN DEVELOPED OVER THE PAST MONTHS IS POISED TO GUIDE CIRM IN MAKING INFORMED DECISIONS AROUND THE ALLOCATION OF OUR REMAINING RESOURCES.

SO THE NEXT PART OF OUR PRESENTATION IS WHAT IS THIS STRATEGIC ALLOCATION FRAMEWORK? WHAT DOES IT CONSIST IN? AND HOW HAS IT BEEN MANDATED? THE MANDATE FOR THE STRATEGIC ALLOCATION FRAMEWORK ORIGINATED AT THE SCIENCE SUBCOMMITTEE OF SEPTEMBER OF 2023. IN THAT MEETING BOARD MEMBER MARK FISCHER-COLBRIE KICKED OFF A PRIORITIZATION DISCUSSION IN WHICH THE NEED FOR A STRATEGIC ALLOCATION PLAN WAS INTRODUCED.

DURING THAT MEETING THE BOARD ASKED CIRM STAFF TO DEVELOP AN APPROACH AND RECOMMENDATIONS FOR PRIORITIZATION. AND THIS IS WHAT WE'VE BEEN DOING OVER THE PAST MONTHS.

THE FRAMEWORK WAS THEN PRESENTED AT THE MARCH ICOC MEETING, THIS PAST MARCH. AND IT REFLECTS A COMPREHENSIVE STRATEGY DEFINING THE

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RATIONALE, THE OBJECTIVES, THE SCOPES, AND TIMELINE NECESSARY TO ESTABLISH CIRM'S FUTURE COURSE OF ACTION. THE STRATEGIC ALLOCATION FRAMEWORK IS STRUCTURED AND DATA DRIVEN -- THAT'S VERY IMPORTANT. WE ARE COLLATING A LOT OF DATA -- AND FROM INPUT FROM MANY DIFFERENT KOL'S.

IT IS A DATA DRIVEN APPROACH TO PRIORITIZE RESOURCE ALLOCATION AND PROVIDE FURTHER GRANULARITY IN TERMS OF IMPACT GOALS AND THEIR SUCCESS MEASURES ULTIMATELY LEADING TO RECOMMENDATIONS THAT WILL COME IN SEPTEMBER TO THE BOARD FOR CONTINUED IMPLEMENTATION OF OUR STRATEGIC PLAN.

NOW, AS WE PRESENTED DURING THE MARCH ICOC, THIS SLIDE PRESENTS THE PIVOTAL DESIGN QUESTIONS AT THE HEART OF OUR STRATEGIC ALLOCATION FRAMEWORK THAT WILL GUIDE OUR FUTURE IMPACT AND RESOURCE DISTRIBUTION. OUR TASK IS TO FIGURE OUT HOW WE CAN USE CIRM'S RESOURCES TO MAKE THE BIGGEST DIFFERENCE. AND IN ADDRESSING THESE QUESTIONS, WE ARE NOT JUST PLANNING FOR THE NEXT COUPLE OF YEARS. WE ARE LAYING DOWN A ROADMAP FOR CIRM THAT WILL EXTEND ITS INFLUENCE FAR INTO THE FUTURE, ESTABLISHING CIRM'S LEGACY.

THE FOLLOWING IS A REMINDER OF WHAT YOU'VE ALREADY SEEN AT THE MARCH ICOC. THIS IS THE PROCESS

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THAT OUR TEAM IS INTEGRALLY INVOLVED FOR THE STRATEGIC ALLOCATION FRAMEWORK. THE BOARD DOES A LOT OF ITS HEAVY LIFTING THROUGH THE SCIENCE SUBCOMMITTEE, THE NEURO TASK FORCE, AND THE ACCESSIBILITY AND AFFORDABILITY WORKING GROUP.

IN THE COMING MONTHS TO THE SEPTEMBER ICOC IN WHICH WE WILL BE PRESENTING THE FINAL RECOMMENDATIONS, THE BOARD, THROUGH THESE DIFFERENT BODIES WILL BE ACTIVELY -- WILL HAVE BEEN ACTIVELY BEEN COLLABORATING WITH US AND PROVIDING FEEDBACK ON THE DIFFERENT ASPECTS OF THE STRATEGIC ALLOCATION RECOMMENDATIONS AND THE GOALS LEADING TO THE MEETING IN SEPTEMBER.

AND THIS IS CONSISTENT WITH THE WAY THAT WE DEVELOP POLICIES AND OTHER PROCESSES. IT IS A BIT OF A HEAVY LIFT THIS TIME, BUT IS ENTIRELY IN KEEPING WITH THE WAY THAT THINGS HAVE BEEN DONE SO FAR.

SO THE PROCESS BEGAN FOR US WITH DEFINING CIRM'S IMPACT GOALS WITH MEASURABLE SUCCESS METRICS. THESE GOALS ARTICULATE THE DESIRED OUTCOMES AND MILESTONES THAT WE AIM TO ACHIEVE. THESE GOALS ARTICULATE THE OUTCOMES THAT WE DESIRE AND THE MILESTONES THAT WE AIM TO ACHIEVE, ENSURING THAT EVERY DOLLAR ALLOCATED WILL MOVE US CLOSER TO OUR

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VISION. THEY SERVE AS THE FOUNDATIONAL FRAMEWORK FOR THE RECOMMENDATIONS.

THESE GOALS WERE FRAMED ALSO IN THE CONTEXT OF FOUR CATEGORIES. AND WE WILL SEE BOTH, THE CATEGORIES AND THE IMPACT GOALS. FOLLOWING THE MANDATE TO DEVELOP A PRIORITIZATION STRATEGY IN SEPTEMBER, THE TEAM AT CIRM DISTILLED FOUR CATEGORIES THAT ARE ALIGNED WITH THE EXISTING STRATEGIC PLAN AND PROP 14'S AREAS OF FOCUS. AND THEN FROM THOSE CATEGORIES, WE DERIVED THE GOALS. IT IS IMPORTANT TO RECOGNIZE THAT SOME OF THE GOALS AS INITIALLY DEFINED ARE EVOLVING THROUGH THIS PROCESS.

FROM THERE, ONCE WE HAVE DEFINED THE CATEGORIES AND THEN THE IMPORTANT IMPACT GOALS, WE STARTED ASKING WHAT WERE THE QUESTIONS THAT WE NEEDED TO ANSWER IN ORDER TO ACHIEVE THOSE GOALS. AND ONCE WE HAD THOSE QUESTIONS, WE HAD TO ELUCIDATE WHAT WAS THE OPTIMAL AMOUNT AND NATURE OF DATA THAT WE NEEDED TO COLLECT AND WHO DID WE NEED TO INVOLVE IN THAT DATA COLLECTION AND ANALYSIS IN ORDER TO ANSWER THOSE QUESTIONS.

SO ONCE WE HAVE THE DATA COLLECTION AND ANALYSIS, WHICH IS WHERE WE ARE NOW, WE'VE BEEN REDEFINING THOSE IMPACT GOALS. AND THEN YOU GO BACK

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TO SOMETIMES HAVING TO COLLECT A FEW MORE DATA TO FINALIZE THE DEFINITION OF THE GOALS. SO WE ARE RIGHT NOW IN THAT VERY SAME -- AT THIS MOMENT IN THE PROCESS. AND THEN, FINALLY, WE WILL BE COMING TO SEPTEMBER WITH OUR RECOMMENDATIONS TO THE BOARD.

NOW, FOR EACH ONE OF THE GOALS, WE WILL BE MEETING WITH THE NEURO TASK FORCE, SCIENCE SUBCOMMITTEE, OR ACCESSIBILITY AND AFFORDABILITY WORKING GROUP TO DISCUSS THE GOALS, DATA RECOMMENDATIONS AT DIFFERENT POINTS BETWEEN NOW AND SEPTEMBER.

AS I MENTIONED, THESE ARE FOLLOWING THE MANDATE TO DEVELOP A PRIORITIZATION STRATEGY WITH STILL FOUR CATEGORIES THAT ARE ALIGNED WITH EXISTING STRATEGIC PLAN AND PROP 14'S AREAS OF FOCUS. AND THEY ARE ALIGNED WITH THE IMPACT GOALS. IN FACT, THE GOALS ARE WHAT DERIVED FROM EACH ONE OF THESE CATEGORIES. SO AS YOU WILL SEE IN THE NEXT SLIDE, THIS SLIDE SHOWS OUR WORKING HYPOTHESIS THAT IS REPRESENTED IN THIS SET OF PRELIMINARY IMPACT GOALS. WE ARE PRESENTING THIS TO YOU AS PART OF A DISCUSSION OF THE PROCESS THAT WE ARE UNDERTAKING TO GIVE AN INDICATIVE IDEA OF WHAT WE ARE THINKING OF AND WILL BE COMING BACK WITH THE MORE DEFINED GOALS INFORMED BY THE DATA THAT WE'VE BEEN COLLECTING AS I



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WAS MENTIONING EARLIER.

THE PROCESS IS TO REFINE THE GOALS, AND WE ARE UNDERGOING THIS PROCESS AS WE SPEAK. SO FOR THE FIRST CATEGORY OF CELL AND GENE THERAPY APPROVALS, WE HAVE DEFINED TWO GOALS. AND, AGAIN, THIS IS JUST AN INITIAL DRAFT. ONE COULD BE RELATED TO RARE DISEASES, ADVANCE AT LEAST X RARE DISEASE PROJECTS TO BLA, PROPEL X THERAPIES TARGETING DISTINCT PREVALENT DISEASES IN CALIFORNIA TO LATE STAGE TRIALS, INCLUDING A NEUROLOGICAL CONDITION, TO SIGNIFICANTLY REDUCE MORBIDITY AND MORTALITY.

THEN WE HAVE ONE THAT IS FOCUSED ON THE ACCESS AND AFFORDABILITY MANDATE OF CIRM-FUNDED CELL AND GENE THERAPIES. ANOTHER ONE THAT'S MORE FOCUSED ON OUR DISCOVERY EFFORTS AND PILLAR IS DISCOVERY TO TRANSLATION. AND THE LAST GOAL HAS TO DO WITH OUR DIVERSE WORKFORCE DEVELOPMENT MANDATE.

THIS IS AN UPDATED TIMELINE SHOWING THE ICOC, THE SCIENCE SUBCOMMITTEE, AND NEURO TASK FORCE MEETINGS SCHEDULED FROM NOW TILL SEPTEMBER WHEN THE STRATEGIC ALLOCATION FRAMEWORK RECOMMENDATIONS WILL BE COMING TO THE BOARD. AS A REMINDER, THE STRATEGIC ALLOCATION PROCESS WE PRESENTED TO THE MARCH SCIENCE SUBCOMMITTEE IN MARCH. AND SINCE THEN WE HAVE THE FORMATION OF THE STRATEGIC ALLOCATION

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FRAMEWORK TEAM, ANALYSIS GROUP, AND THEN WE STARTED UNDER CIRM'S LEADERSHIP GUIDANCE AND IN COLLABORATION WITH THE BOARD TO COLLECT DATA THAT WILL SUPPORT THE RECOMMENDATIONS THAT WILL COME TO THE BOARD IN SEPTEMBER.

BETWEEN NOW AND SEPTEMBER, WE PLAN ON HAVING -- I'LL SHOW IT AT THE END -- DIFFERENT MEETINGS THAT WILL BE FOCUSING ON THE DIFFERENT GOALS SO WE CAN GATHER SPECIFIC FEEDBACK FROM THE BOARD MEMBERS IN PREPARATION FOR THE FINAL ICOC.

WHAT I WANT TO POINT OUT HERE IS THAT BASICALLY THE ICOC WILL DEFINITELY NOT BE THE FIRST TIME THAT THE BOARD WILL HAVE HAD A CHANCE TO PROVIDE INPUT IN OUR GOALS. SO I JUST WANTED TO MAKE SURE THAT THIS WAS HIGHLIGHTED HERE.

SO THE SECOND PART OF THIS PRESENTATION, I SAW THIS PRESENTATION IN TWO PARTS. WE SAW THIS PRESENTATION IN TWO PARTS. ONE HAS TO DO ON THE UPDATE ON WHERE ARE WE WITH THE HIGH LEVEL STRATEGIC ALLOCATION FRAMEWORK, AND THEN HOW DOES THE NEURO TASK FORCE FIT INTO THIS. SO WE WILL FOCUS ON THE NEURO MANDATE OF PROP 14 AND HOW THE WORK OF THE NEURO TASK FORCE THAT HAS BEEN LED BY DR. GOLDSTEIN INFORM THE STRATEGIC ALLOCATION FRAMEWORK AND OUR PRIORITIZATION EXERCISE.

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THIS SLIDE SHOWS WHERE THE FOCUS OF THE NEURO TASK FORCE RECOMMENDATIONS WILL FALL WITHIN THE CONTEXT OF THE FOUR CATEGORIES THAT WE DEFINED AND BY EXTENSION INFORMING THREE OF THE IMPACT GOALS AND WILL BE INFORMING PROP 14'S AT LEAST 27 PERCENT EARMARK FOR NEURO THAT WILL BE DELINEATED UNDER THE GOALS FOR CATEGORIES 1 AND 3.

AT THE MOMENT WE ARE SPENDING AT A HIGHER RATE, BUT WE HAVE NOT, BESIDES THE REMIND PROGRAM INITIATIVE, WE HAVE NOT DEVELOPED ANY OTHER FOCUS FOR NEURO.

AFTER THE REMIND CONCEPT WAS APPROVED, THE NEURO TASK FORCE ENGAGED, AGAIN UNDER THE LEADERSHIP OF DR. GOLDSTEIN, IN A SERIES OF EXPERT EDUCATIONAL SESSIONS THAT WERE VERY HELPFUL. THE SESSIONS HAD THE GOAL OF PROVIDING AN OVERVIEW OF NEURODEGENERATIVE RESEARCH FIELD, SPOTLIGHTING INNOVATIVE APPROACHES, AND DELINEATING UNDEREXPLORED AREAS AND NEEDS IN THE FIELD. TO ENHANCE AND EXPEDITE THE EXPLORATION OF NOT ONLY NEURODEGENERATIVE DISEASE AREAS, BUT ALL NEURO WITHIN THE STRATEGIC ALLOCATION FRAMEWORK TIMELINE, THE CIRM TEAM HAS CARRIED OUT THE COMPREHENSIVE SURVEY. WE DESIGNED IT WITH THE HELP AND INPUT FROM THE NEURO TASK FORCE MEMBERS AS WELL AS KOL'S. AND

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THIS METHOD ALLOWS US FOR AN IN-DEPTH ANALYSIS AND BROADER STAKEHOLDER ENGAGEMENT, PROVIDING A QUICKER, MORE EXTENSIVE UNDERSTANDING OF CURRENT RESEARCH LANDSCAPE AND FUTURE DIRECTIONS THAT ALIGNS ALSO WITH THE TIME FOR THE STRATEGIC ALLOCATION FRAMEWORK.

THE SURVEY DESIGN PIVOTED FROM THE COMPREHENSIVE DESIGN BRIEF DEVELOPED DURING THE EDUCATIONAL SESSIONS, AND THE SURVEY QUESTIONS WERE DESIGNED BASED ON THIS DESIGN BRIEF. AND WE COVERED THESE FOUR AREAS, IDENTIFYING BOTTLENECKS AND KNOWLEDGE GAPS THAT COULD UNIQUELY BENEFIT FROM MULTIDISCIPLINARY SOLUTIONS AND KNOWLEDGE SHARING, FINDING CROSS-DISEASE ANALYSIS, DISCUSSING HOW INSIGHTS FROM STEM CELL AND GENETIC RESEARCH IN NOT ONLY ONE NEURODEGENERATIVE DISEASE, BUT ANY OTHER NEURODISEASE CAN BE APPLIED TO OTHERS, AND DISCUSSED HOW INSIGHTS AND INNOVATIVE TOOLS AND TECHNIQUES CAN BE APPLIED ACROSS DISEASES, AND THEN DISCUSSING A POTENTIAL ROLE FOR CIRM IN ADDRESSING THESE ABOVE POINTS.

SO I'M NOW GOING TO BE FOCUSING ON SELECT DATA THAT WE HAVE FOUND THAT IS VERY RELEVANT FOR INFORMING THE STRATEGIC ALLOCATION FRAMEWORK. THIS IS A SUMMARY OF THE TOTAL SURVEY RESPONSES. CIRM

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CONTACTED 679 NEUROSCIENTISTS, THEY WERE PH.D. AS WELL AS CLINICIANS, INVITING THEM TO PARTICIPATE IN OUR SURVEY TO PROVIDE THEIR EXPERT OPINIONS AND FEEDBACK.

OUT OF THOSE CONTACTED, 136 STARTED FILLING OUT THE SURVEY, SHOWING AN INITIAL ENGAGEMENT OF ABOUT 20 PERCENT. THIS METRIC HELPS US UNDERSTAND THE INTEREST LEVEL AMONGST THE NEURO COMMUNITY REGARDING THE TOPICS THAT WE ARE EXPLORING.

OF THOSE, 111 COMPLETED THE ENTIRE SURVEY. THIS GIVES US A SUBSTANTIVE COMPLETION RATE OF ABOUT 16 PERCENT FROM THE INITIAL CONTACTS, AND THIS RESPONSE RATE IS QUITE INSIGHTFUL, INDICATING THE WILLINGNESS OF THE COMMUNITY TO CONTRIBUTE TO OUR UNDERSTANDING OF CRITICAL ISSUES IN THE NEURO FIELD AND REGENERATIVE MEDICINE.

EACH OF THOSE 111 RESPONSES IS PACKED WITH VALUABLE INSIGHTS FROM FIELD EXPERTS, AND THEY ARE INSTRUMENTAL IN SHAPING OUR INITIATIVES MOVING FORWARD.

WE ARE GOING NOW DIG DEEPER INTO THE RESULTS. I WANTED TO COMMENT AS WELL THAT WE GATHERED ALL THE NEURO EXPERTS THAT WE KNEW BETWEEN ALL THE APPLICANTS THAT HAVE EVER APPLIED TO CIRM IN

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THE NEURO FIELD AS WELL AS GRANTEES AS WELL AS ANYBODY ELSE THAT WE CAN FIND IN CALIFORNIA. SO IT'S HEAVY SKEWED ON CALIFORNIA RESEARCHERS, BUT WE THOUGHT THAT THIS WAS VERY IMPORTANT.

SO TO BETTER UNDERSTAND THE CONTEXT OF THE FEEDBACK WE RECEIVED, IT'S CRUCIAL TO CONSIDER WHO OUR RESPONDENTS ARE. AND AS I WAS MENTIONING, A SIGNIFICANT MAJORITY OF OUR RESPONDENTS, 60 PERCENT, ARE BASED IN CALIFORNIA, WHICH IS CIRM'S FOCUS. WE REACHED OUT TO OUR WHOLE PORTFOLIO, AS I WAS SAYING, AND AWARDEES AND APPLICANTS OVER CIRM'S HISTORY.

ADDITIONALLY, ONE-THIRD OF OUR RESPONDENTS COME FROM VARIOUS OTHER PARTS OF THE UNITED STATES WHILE 7 PERCENT REPRESENT INTERNATIONAL PERSPECTIVES, ADDING A GLOBAL DIMENSION TO THE DATA THAT WE HAVE COLLECTED.

MOST OF OUR RESPONDENTS, 84 PERCENT, ARE FROM ACADEMIC INSTITUTIONS, WHICH UNDERSCORES THE SURVEY RESPONSES HAVE STRONG ACADEMIC ORIENTATION. AND MEANWHILE 6 PERCENT OF OUR RESPONDENTS ARE FROM INDUSTRY, WHICH INDICATES VALUABLE INPUT FROM THE PRIVATE SECTOR. AND 9 PERCENT ARE AFFILIATED WITH NON-PROFIT OR GOVERNMENT ORGANIZATIONS, WHICH HELPS ROUND OUT THE PERSPECTIVE WE HAVE CAPTURED. I MUST SAY THAT FROM NON-PROFIT/GOVERNMENT, WE HAD HEADS OF

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NIH INSTITUTES RELATED TO NEURO THAT RESPONDED TO THIS SURVEY, WHICH IS PRETTY ENCOURAGING AND SUPPORTIVE OF THEM AS WELL.

THIS SLIDE REPRESENTS A DETAILED BREAKDOWN OF THE NEURO DISEASE DISORDERS SURVEY RESPONSES. ALZHEIMER'S DISEASE IS THE MOST FREQUENTLY REPORTED CONDITION IN THE 22 RESPONSES. THAT MEANS THAT OF THE PEOPLE SURVEYED, 22 WERE RESPONSES FROM ALZHEIMER'S EXPERTS. AND THIS WAS SOMETHING THAT -- THIS DOES NOT MEAN THAT THESE ARE THE PREVALENT OR NONPREVALENT DISEASES. IT KIND OF ALIGNS A LITTLE BIT; BUT, FOR EXAMPLE, STROKE IS THE MOST PREVALENT NEURO DISEASE IN CALIFORNIA. SO I JUST WANT TO MAKE SURE THAT IT'S NOT CONFUSED BY THAT. BUT THE TREND UNDERSCORES THE ONGOING CONCERN AND RESEARCH FOCUS WITHIN THESE AREAS AT LEAST FROM THE RESPONDENTS.

LOOKING AT THE GEOGRAPHICAL DATA, WE SEE A SIGNIFICANT CONCENTRATION OF RESPONSES FROM CALIFORNIA, WHICH IS LOGICAL BECAUSE THAT'S PEOPLE THAT WE HAD TARGETED THE MOST. AND FOR SOME DISEASES IT'S MORE, LIKE AD AND OTHER NEURODEGENERATIVE DISEASES. HOWEVER, CONDITIONS LIKE AUTISM SPECTRUM DISORDERS SHOW A MORE DISTRIBUTED PATTERN ACROSS THE U.S. REGION AND INTERNATIONAL, SUGGESTING BROADER INTEREST AND

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VARIABILITY IN RESEARCH LOCATIONS.

AND LASTLY, INSTITUTIONALLY A VAST MAJORITY OF THE RESPONSES COME FROM ACADEMIC SETTINGS AS WE HAD ALREADY SEEN. SO THIS MULTIDIMENSIONAL ANALYSIS ALLOWS US TO UNDERSTAND NOT ONLY THE FREQUENCY OF THE CONDITIONS THAT WE GOT ANSWERS FOR, BUT ALSO WHERE AND BY WHOM THE DATA WAS REPORTED TO HAVE A BIT OF A CONTEXT OF WHAT WE ARE GOING TO SEE.

NOW, THE NEXT SET OF DATA IS GOING TO HAVE TO DO WITH THE DISCOVERY/TRANSLATIONAL KNOWLEDGE GAPS AND THEN CLINICAL DEVELOPMENT BOTTLENECKS. SO I'M GOING TO TALK ABOUT WHAT WE RECEIVED IN TERMS OF THOSE TWO ASPECTS.

FOR THE FIRST QUESTION, IN TERMS OF COMMON KNOWLEDGE GAPS IN DISCOVERY AND TRANSLATION, WE ASKED EACH EXPERT TO IDENTIFY THREE CRITICAL KNOWLEDGE GAPS IN THE UNDERSTANDING OF THEIR DISEASE AREA THAT, IF ADDRESSED, COULD HELP ACCELERATE THE FIELD FORWARD IN THE NEXT FIVE YEARS. THE MOST PROMINENT GAP, AS YOU CAN SEE, IS UNDERSTANDING DISEASE MECHANISMS WITH 86 RESPONSES, EMPHASIZING THIS AREA OF NEED AND INDEPENDENT OF DISEASE IN THIS CASE.

THIS IS FOLLOWED BY GAPS IN TRANSLATIONAL AND CLINICAL DEVELOPMENT. SO EVEN IF WE WERE ASKING



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ABOUT DISCOVERY TRANSLATION, THAT CAME UP. THERE ARE GAPS IN TRANSLATIONAL AND CLINICAL THAT NEED TO BE TACKLED. AND BIOMARKERS, SUGGESTING A PRESSING NEED TO BRIDGE THE GAP BETWEEN BASIC RESEARCH AND CLINICAL APPLICATIONS.

OTHER SIGNIFICANT AREAS NEEDING ATTENTION INCLUDE FOUNDATIONAL BIOLOGY AND THE NEURO-IMMUNE AXIS, WHICH IS BECOMING INCREASINGLY RELEVANT IN OUR UNDERSTANDING OF NEURODISEASES. AND THESE AREAS ARE CRITICAL FOR DEVELOPING TARGETED THERAPIES AND IMPROVING DIAGNOSTIC ACCURACY.

NOW, THIS SLIDE FURTHER CATEGORIZES THE SAME RESPONSES INTO STAGES OF RESEARCH FROM DISCOVERY, TRANSLATION, AND CLINICAL, HELPING US PINPOINT EXACTLY WHERE IN THE RESEARCH PIPELINE THE GAPS ARE MORE SIGNIFICANTLY BASED AND UNDERSURVEYED RESPONDENTS. AND WHAT WE CAN SEE IS THAT MOST OF THEM ARE IN THE DISCOVERY AND TRANSLATION WITH ONE OF THEM BEING CLINICAL STAGE AND BEYOND.

NOW, THIS MATRIX, THIS TABLE REPRESENTS THE SAME -- IS SHOWING US THE SAME DATA ACROSS THE SPECIFIC NEURODISEASES. EACH COLUMN REPRESENTS A SPECIFIC CONDITION WHILE EACH ROW DENOTES AN AREA WHERE KNOWLEDGE GAPS HAVE BEEN IDENTIFIED FROM WHAT WE'VE SEEN IN THE OTHER TWO

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SLIDES, WHICH SHOWS US CLEAR, VISUAL REPRESENTATIONS OF WHERE OUR FOCUS COULD GO.

SO FOR DISEASE MECHANISMS, IT WAS ACROSS MOST OF THE DISEASES THAT WE RECEIVED AN ANSWER FOR THAT PINPOINTED DISEASE MECHANISMS AND ONE OF THE MOST COMMON KNOWLEDGE GAPS. THAT DOESN'T MEAN THAT CANCER OF THE BRAIN DOES NOT HAVE DISEASE MECHANISM KNOWLEDGE GAPS. IT MEANS THAT WE DID NOT RECEIVE THAT AS AN ANSWER, BUT IT'S GIVING A LITTLE BIT OF A HELP FOR US TO SEE WHERE WE SHOULD FOCUS AT LEAST IN TERMS OF KNOWLEDGE GAPS AGNOSTICALLY OF EVEN DISEASES.

NOW, I'M GOING TO MOVE INTO THE SECOND MAIN QUESTION THAT WE ASKED WHICH HAD TO DO WITH COMMON BOTTLENECKS FOR DEVELOPMENT OF NEUROTHERAPIES.

SO WE ASKED HERE FOR EACH EXPERT TO PROVIDE THREE DEVELOPMENT BOTTLENECKS IN THE DEVELOPMENT OF THE THERAPY FOR THE DISEASE THAT THEY ARE FOCUSING ON. AND THE MOST NOTABLE BOTTLENECK, AGAIN, WITH 64 RESPONSES RELATES TO MECHANISTIC GAPS, INDICATING A CRITICAL NEED FOR DEEPER UNDERSTANDING OF THE UNDERLYING MECHANISMS OF NEUROLOGICAL DISORDERS. THIS IS FOLLOWED BY CHALLENGES IN PRECLINICAL MODELS AND LACK OF

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BIOMARKERS, HIGHLIGHTING BARRIERS IN EARLY STAGE RESEARCH THAT IMPACT THE ENTIRE DEVELOPMENT PIPELINE. AND THEN THE SAME TYPE OF ANALYSIS SHOWING CATEGORIZATION OF THE RESPONSES INTO STAGES FROM DISCOVERY, TRANSLATIONAL, AND CLINICAL, PINPOINTING THAT A LOT OF, EVEN WHEN WE ARE ASKING ABOUT THE BOTTLENECKS IN DEVELOPMENT OF THERAPIES, WHAT WE ARE GETTING IS STILL DISCOVERY AND TRANSLATION AND THEN OBVIOUSLY CLINICAL ISSUES.

NOW, THIS POINTS TO A COUPLE POINTS I WANT TO MAKE HERE. OBVIOUSLY MANY WERE ACADEMIC RESPONDENTS, SO THEIR RESEARCH IS MORE BASIC. AND ALSO IT POINTS OUT THAT THE READINESS OF THE FIELD, THERE ARE NOT AS MANY ISSUES IDENTIFIED IN MANUFACTURING BESIDES SOME PARKINSON'S RESEARCHER THAT PROVIDED THOSE RESPONSES. THE FIELD IS IN NEED FOR EARLIER INVESTMENT IN BASIC RESEARCH AND TRANSLATIONAL BOTTLENECKS. THAT'S WHAT WE'VE BEEN HEARING.

THIS SLIDE SHOWS IN TERMS OF THE DISEASES, HOW THESE DEVELOPMENT BOTTLENECKS SPREAD ACROSS THE DISEASES. AGAIN, ALL THESE SLIDES HAVE BEEN SHARED AND THEY ARE PART OF THE MATERIALS.

LASTLY, WE WOULD LIKE TO SHOW, GIVEN THAT ALL THIS DATA WAS POINTING TO EARLIER RESEARCH

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MECHANISMS AND TRANSLATIONAL BOTTLENECKS, ESPECIALLY FOR EARLY RESEARCH MECHANISMS, WE NEED TO USE STEM CELL MODELS. THAT'S SOMETHING THAT DR. GOLDSTEIN, WHEN WE STARTED WITH THE NEUROPSYCHIATRIC FOCUS, WANTED TO QUERY. WE INVITED SPEAKERS TO FIGURE OUT WHETHER THE READINESS OF THE STEM CELL MODELS AND GENETICS WERE AT THE LEVEL THAT WE NEEDED IN ORDER TO INVEST IN EARLY RESEARCH FOR THAT.

SO WE QUERIED -- IT WAS PART OF THE SURVEY, HOW WE HAD DESIGNED IT. SO THE RESPONSES HAVE BEEN POINTING TO MECHANISTIC UNDERSTANDING DISCOVERY OF BIOMARKERS. SO WE SET TO ANALYZE WHAT RESPONDENTS HAD SAID ABOUT THE CURRENT USE OF STEM CELL MODELS WHICH ARE CRUCIAL TOOLS IN THE STUDY OF THESE DISEASES. AND WE AVERAGED THEIR RESPONSES IN TERMS OF CURRENT EFFECTIVENESS.

THE SLIDE PROVIDES A SNAPSHOT OF HOW THESE MODELS ARE CURRENTLY BEING UTILIZED ACROSS A RANGE OF NEURODISORDERS. AND, ALZHEIMER'S, FOR EXAMPLE, THE MODELS ARE EFFECTIVE FOR BASIC DISEASE MODELING, BUT FALL SHORT IN DRUG SCREENING. THE EFFECTIVENESS IS COMPILATION OF MANY ANSWERS THAT WE SUMMARIZED IN ONE SENTENCE. IT'S NOT JUST ONE SINGLE ANSWER, JUST A SUMMARY OF WHAT WE GATHERED.

IN CONTRAST, FOR PARKINSON'S DISEASE,

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WHILE GENERALLY EFFECTIVE, THEY STRUGGLE WITH MODELING AGE-RELATED CHANGES, A CRITICAL ASPECT OF THE DISEASE. AND WE ACTUALLY HEARD THAT FROM DR. LORENZ STUDER WHEN HE PROVIDED -- IN THE EDUCATIONAL SESSION, THAT WAS ONE OF THE ASPECTS THAT HE TACKLED.

IN DISEASES LIKE ALS AND HUNTINGTON'S DISEASE, THERE'S NOTABLE DISAGREEMENT ON THE EFFECTIVENESS OF STEM CELL MODELS. THIS VARIABILITY UNDERSCORES THE COMPLEXITY OF THESE DISEASES AND THE CHALLENGES IN MODELING THEM ACCURATELY. THAT DOESN'T MEAN THAT THEY ARE NOT USING THE MODELS. THEY ARE BEING USED WHICH MEANS THAT THEY ARE USEFUL FOR SOME THINGS, BUT IT ALSO MEANS THAT WE NEED TO INVEST IN THAT.

SO WITH THAT, I'M JUST GOING TO MOVE NOW INTO THE HIGH LEVEL NEEDS THAT DERIVED FROM THE ANSWERS FROM THE SURVEY. THIS SLIDE SYNTHESIZES CRITICAL INSIGHTS THAT WE GATHERED FROM OUR EXTENSIVE SURVEY, HIGHLIGHTING THE HIGH LEVEL NEEDS IN NEURORESEARCH DERIVED FROM THE RESPONDENTS. THE SURVEY POINTS TO A NEED TO INVEST IN FOUNDATIONAL DISCOVERY INITIATIVES. RESPONSES HIGHLIGHT AN URGENT NEED TO BETTER UNDERSTAND THE FUNDAMENTAL MECHANISMS IN NEURODISEASES ACROSS ALL INDICATIONS,

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BROAD ACKNOWLEDGEMENT OF THE GROWING UTILITY AND IMPORTANCE OF HUMAN STEM CELL MODELS, AND THE NEED TO CONTINUE IMPROVEMENTS IN THIS AREA. AND RESPONSES ALSO HIGHLIGHTED COMMON AREAS OF POTENTIAL INVESTMENT THAT COULD HAVE BROAD IMPACT.

AND IN EFFICIENT DISCOVERY TO TRANSLATION. SO WE ALSO HEARD THAT WE NEED TO BE MORE EFFICIENT ON THE DISCOVERY TO TRANSLATION PATH. SO THE HIGH LEVEL NEEDS ARE IN INNOVATION IN CELL AND GENE THERAPY TECHNOLOGIES AND IMPROVE UNDERSTANDING OF THERAPEUTIC MECHANISMS AGAIN. THE DELIVERY AND TARGETING TO THE BRAIN AND SPECIFIC CELL TYPES. THIS IS SOMETHING THAT JUST KEEPS COMING, AND WE ARE HEARING IT ALL OVER.

NEW BIOMARKER IDENTIFICATION AND VALIDATION, ESPECIALLY THOSE THAT ADDRESS DISEASE HETEROGENEITY AND PATIENT CERTIFICATION AS WELL AS EARLY (UNINTELLIGIBLE) DIAGNOSIS.

OTHER BOTTLENECKS IN CELL AND GENE THERAPY TRANSLATION AND CLINICAL DEVELOPMENT ALSO INCLUDE TREATMENT DURABILITY AND TRANSPLANT SURVIVAL AS WELL AS SCALE AND QUALITY CONTROL IN MANUFACTURING.

NOW, ALL THOSE POINTS ARE DISCOVERY AND TRANSLATION. AND THE GOAL OF TODAY'S MEETING WAS TO COMPLETE THE EXERCISE OF THE NEURO TASK FORCE

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EDUCATION SESSIONS WITH THE NEURO SURVEY INFORMING THE BOTTLENECKS AND AREAS AND GAPS THAT WE SEE IN THE NEURO FIELD.

NOW, DRAWING ON EXTENSIVE SURVEY FEEDBACK, WE HAVE IDENTIFIED PRELIMINARY RECOMMENDATIONS TO ADDRESS TWO CRITICAL AREAS IN NEURORESEARCH. NOW, BEFORE DIGGING INTO THE DETAILS, I WOULD LIKE TO CLARIFY THAT THIS IS JUST ONE. SO THE SURVEY IS JUST ONE, WITH THE EDUCATIONAL SESSIONS, IS ONE OF THREE APPROACHES THAT WILL BE INFORMING OUR IMPACT GOALS IN THE NEURO FIELD. THE OTHER TWO APPROACHES ARE COMPLEMENTARY TO THIS ONE AND ARE STILL UNDERGOING. WITH THAT, WHAT I WOULD LIKE TO MAKE CLEAR IS THAT EVEN IF THE SURVEY RESULTS POINT TO EARLIER RESEARCH NEEDS, WHICH IS A VERY REAL NEED IN THE FIELD, THERE ARE STILL CLINICAL DEVELOPMENT NEEDS AND BOTTLENECKS THAT WILL BE TAKEN INTO ACCOUNT IN THE DISCUSSIONS OVER THE COMING MONTHS. AND I JUST WANTED TO MAKE SURE THAT I MENTIONED THIS BECAUSE IT'S VERY IMPORTANT.

OUR FIRST RECOMMENDATION EMPHASIZES THE NEED TO INCREASE FOUNDATIONAL AND MECHANISTIC DISCOVERY. INCREASING RESEARCH INTO CROSS-DISEASE SYSTEMS AND INTERACTIONS. BY DOING SO, WE AIM TO ACHIEVE BREAKTHROUGHS IN NEURODISEASE MECHANISMS,

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TARGETS, AND BIOMARKERS, WHICH IS WHAT WE'VE BEEN HEARING ALL OVER THROUGH THE SURVEY RESPONSES. AND THERE IS A POTENTIAL TO ACCOMPLISH THESE THROUGH AN INITIATIVE THAT CIRM SPENT A YEAR IN THE MAKING AND THAT THE BOARD PRESSURE TESTED FROM ALL SIDES. AND THIS INITIATIVE COULD ALLOW US TO PROMOTE COLLABORATIVE, MULTIDISCIPLINARY INNOVATION IN STEM CELL AND GENETIC RESEARCH ACROSS VARIOUS DISCIPLINES AND INDICATIONS WITHOUT THE FOCUS IN A SPECIFIC NEURODISEASE. MORE AROUND A SYSTEM LIKE NEURO-IMMUNE AXIS OR SOMETHING OF THAT KIND. BUT THAT OBVIOUSLY COULD BE MORE AT THE -- WE COULD BE DISCUSSING THIS FURTHER IN THE CONTEXT OF THE NEURO TASK FORCE AND SCIENCE SUBCOMMITTEE AT A LATER MEETING.

THE SECOND COULD BE THAT WE NEED TO INCREASE EFFICIENT DISCOVERY TO TRANSLATION. ENHANCING INVESTMENTS TO ADDRESS SIGNIFICANT COMMON TRANSLATIONAL NEEDS AND BOTTLENECKS ACROSS CELL AND GENE THERAPY SPACE TO ACCELERATE TRANSITION FROM BENCH TO BEDSIDE. THIS TALKS ABOUT THE BOTTLENECKS THAT WE WERE TALKING ABOUT LIKE DELIVERY, TARGETING THE RIGHT THERAPIES, THE RIGHT CELL POPULATIONS AND OTHERS. SO THE POTENTIAL TO ACCOMPLISH THIS IS THROUGH A REVITALIZED STRUCTURE INTEGRATING THE



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DISC2, TRAN, AND CLIN1 PROGRAMS, WHICH COULD AIM TO STREAMLINE PROCESSES THAT IDENTIFY AND TRANSLATE THERAPEUTIC AND BIOMARKER CANDIDATES, WHICH WOULD ALLOW US TO ADDRESS COMMON BOTTLENECKS AND ACCELERATE THE TRANSITION FROM BENCH TO BEDSIDE.

SO THIS BASICALLY COULD BE REVITALIZING THE PILLAR ORGANIZATIONAL STRUCTURE OF OUR TRANSLATIONAL PROGRAM. THESE ARE, AGAIN, JUST PRELIMINARY RECOMMENDATIONS THAT COULD BE COMING TO THE JULY 11TH SCIENCE SUBCOMMITTEE WHEN WE WILL DISCUSS GOAL 4, WHICH IS THE ONE THAT'S FOCUSED MORE ON THE DISCOVERY OF MECHANISMS.

WITH THAT SAID, IN TERMS OF NEXT STEPS, HOW AND WHEN WILL WE BE DISCUSSING ALL THESE RECOMMENDATIONS IN THE OVERALL CONTEXT OF THE FIVE IMPACT GOALS IS COMING IN THE NEXT SLIDE.

SO THIS SLIDE SHOWS WHERE WE ARE TODAY. TODAY WE DISCUSSED THE NEURO TASK FORCE BACKGROUND, THE SURVEY RESULTS, AND HOW DOES THIS FIT INTO THE STRATEGIC ALLOCATION FRAMEWORK AND PRELIMINARY RECOMMENDATIONS. AT THE JUNE ICOC WE WILL BE PROVIDING AN OVERALL UPDATE ON THE PROCESS OF THE NEURO TASK FORCE -- ON THE PROCESS OF THE STRATEGIC ALLOCATION FRAMEWORK AND OFFER AN EXAMPLE OF THE ANALYSIS THAT WILL INFORM THE RECOMMENDATIONS.

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BUT THEN MOVING INTO JULY 11TH, IT'S ACTUALLY THE JULY 11TH JOINT NEURO TASK FORCE/SCIENCE SUBCOMMITTEE, THAT'S WHEN WE WILL BE PRESENTING THE FIRST ONE OF THE GOALS. WE ARE STARTING WITH GOAL 4 BECAUSE IT'S THE GOAL THAT WE HAVE MORE DATA ON, AND IT'S GOING TO BE A GOOD TESTGROUND FOR HOW THESE DISCUSSIONS WILL BE GOING AS WELL. SO WE NEED MORE TIME TO COME TO THE AUGUST NEURO TASK FORCE/SCIENCE SUBCOMMITTEE TO DISCUSS THE CELL AND GENE THERAPY APPROVALS, TWO GOALS, THE ONE ON RARE AND PREVALENT DISEASES.

AND THEN AT THE AUGUST ACCESSIBILITY AND AFFORDABILITY WORKING GROUP, WE WILL BE DISCUSSING GOAL 3 THAT HAS TO DO WITH ACCESSIBILITY AND AFFORDABILITY MANDATE. WE'VE ALREADY PRESENTED THE GOAL TO THE ACCESSIBILITY AND AFFORDABILITY WORKING GROUP, AND WE ARE GATHERING SOME OF THEIR FEEDBACK IN TERMS OF DATA THAT WE NEED TO GATHER, AND WE WILL BE PRESENTING A FINAL RECOMMENDATION HERE.

AND THEN AT THE SCIENCE SUBCOMMITTEE OF SEPTEMBER, NO, THERE IS NOT A NEURO TASK FORCE/SCIENCE SUBCOMMITTEE. IT'S JUST THE FULL SCIENCE SUBCOMMITTEE. THIS IS WHERE WE WILL PRESENT THE FIVE GOALS, INCLUDING THE EDUCATION WORKFORCE DEVELOPMENT GOAL, FOR FINAL DISCUSSION AND FEEDBACK

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TWO WEEKS BEFORE THE FINAL SEPTEMBER ICOC WHERE WILL BE PRESENTING THE FINAL STRATEGIC ALLOCATION FRAMEWORK RECOMMENDATIONS.

AND WITH THAT, I WOULD LIKE TO THANK EVERYBODY FOR THE ATTENTION, AND WE ARE OPEN FOR QUESTIONS.

CHAIRMAN FISCHER-COLBRIE: JUST A QUICK COMMENT. I THINK WE'VE GOT A WHOLE LIST OF POSSIBLE QUESTIONS. ONE IS CATCHING UP ON THE INFORMATION PROVIDED. THE SECOND HAS TO DO WITH PROCESS QUESTIONS AROUND WHAT IS GOING TO BE NEEDED TO BE REQUIRED TO MOVE THIS FORWARD. AND THE THIRD OBVIOUSLY RELATED TO ANY OF THE SPECIFIC OUTLINES OF A WORKING DRAFT FROM THE GOALS AND/OR THE INFORMATION PROVIDED ON A MORE GRANULAR BASIS.

SO I THINK IT'S FAIRLY OPEN FROM THAT PERSPECTIVE. BEFORE WE LAUNCH INTO THAT DIALOGUE, J.T., I DON'T KNOW IF THERE'S OTHER QUESTIONS OR OTHER FRAMING YOU'D LIKE TO PROVIDE.

DR. CANET-AVILES: NO. THIS IS IT. THANK YOU.

CHAIRMAN FISCHER-COLBRIE: OKAY. ALL RIGHT. WITH THAT, I THINK I SAW FRED FISHER'S HAND POP UP. FRED, I THINK YOU'RE ON MUTE.

DR. FISHER: SORRY. SO WHAT JUMPS OUT --

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I MEAN THERE'S A LOT TO DIGEST HERE FOR SURE. SO MY FIRST BASIC QUESTION IS THE SEPTEMBER MEETING IS NOT A JOINT MEETING. AND SO NEURO TASK FORCE PEOPLE WON'T BE A PART OF THAT MEETING, BUT IT SEEMS LIKE EXTREMELY RELEVANT DATA WILL BE PRESENTED AT THAT MEETING. AND SO I'M WONDERING ABOUT WHY THE EXCLUSION OF THE NEURO TASK FORCE FROM THAT PARTICULAR MEETING.

DR. CANET-AVILES: THANK YOU, FRED.  
ANYBODY WANTS TO ANSWER? I'M HAPPY TO ANSWER, BUT ANYBODY ELSE WOULD LIKE TO ANSWER?

MR. TOCHER: HI, FRED. THIS IS SCOTT.  
I'M NOT SURE IF THAT WAS INTENTIONAL OR INADVERTENT, BUT TO GET TO THE ANSWER IS WE CAN ALWAYS MAKE THAT ANOTHER JOINT MEETING AT THE END OF THE DAY. SO IF THAT'S YOUR DESIRE, THAT'S ABSOLUTELY NOT A PROBLEM.

DR. FISHER: I THINK IT MAKES SENSE JUST FOR CONTINUITY OF THE PROCESS.

DR. CANET-AVILES: YES.

CHAIRMAN IMBASCIANI: THANK YOU, SCOTT.  
YOU BEAT ME TO IT. I WAS GOING TO SUGGEST THE SAME.

MR. TOCHER: GREAT. WE'LL DO THAT.

DR. FISHER: THANK YOU.

CHAIRMAN IMBASCIANI: THAT SITS WELL WITH YOU, FRED?

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DR. FISHER: YEAH. I THINK IT MAKES SENSE TO HAVE IT BE ANOTHER JOINT MEETING GIVEN THE CONTENT THAT'S BEING PRESENTED AND CONTINUITY OF THE PROCESS. SO THANK YOU FOR DOING THAT.

CHAIRMAN FISCHER-COLBRIE: OKAY. I DON'T KNOW IF I HAVE IT RIGHT IN TERMS OF SEQUENCE, BUT I SEE PAT LEVITT'S GOT HIS HAND UP.

DR. LEVITT: HEY, MARK. SO, ROSA, THANKS VERY MUCH. A LOT OF REALLY IMPORTANT INFORMATION. THE THING THAT -- AND I WENT THROUGH THE SLIDES BEFORE THE MEETING. THE THING THAT STUCK OUT FOR ME IMMEDIATELY WAS THE DATA ON SLIDE 24, WHICH IS THE RESPONSES BASED ON ACADEMIC, BIOTECH, PHARMA, GOVERNMENT, NON-PROFIT, AND OTHER. IF YOU LOOK AT THAT DATA, THERE ARE 21 DISEASES THAT ARE LISTED, DISEASES OR DISORDERS. AND 5 OUT OF THE 21 HAVE ANY KIND OF AN INDUSTRY RESPONSE, THAT IS BIOTECH, PHARMA, 5 OUT OF 21. ELEVEN OUT OF THE 21 ARE A HUNDRED PERCENT ACADEMIC. AND I THINK IT'S A REFLECTION OF THE OVERWHELMING NUMBER THAT RESPONDED THAT THE MAJOR ROADBLOCKS FOR NEURO REVOLVE AROUND AN UNDERSTANDING OF MECHANISMS.

AND SO ONE THOUGHT I HAD IN TERMS OF AT LEAST A DISCUSSION AND THINKING ABOUT STRATEGIES AND GOALS AND HOW TO APPROACH THIS IS TO FIGURE OUT HOW

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WE CAN, IN TERMS OF WHAT WE DO AT CIRM, INCREASE THOSE WHO ARE IN BIOTECH AND PHARMA, WHICH ARE INCREDIBLY IMPORTANT PARTNERS, AS CERTAINLY THAT'S THE CASE IN OTHER DISEASES THAT CIRM IS TACKLING, THAT CIRM AWARDEES ARE TACKLING IN CANCER, HEART DISEASE, DIABETES, IMMUNE DISORDERS, ET CETERA. BECAUSE TO ME THIS IS THE MAJOR CHALLENGE FOR US IN TERMS OF GOING FORWARD, TO TRY FIGURE OUT HOW TO GET THOSE INCREASED PARTNERSHIPS OR THE RECOGNITION THAT WHAT CIRM IS DOING IS GOING TO BE IMPORTANT FOR THE TRANSLATIONAL PART.

SO I'LL STOP THERE. I THINK THIS SLIDE NO. 24 IS LIKE IT'S TRANSPARENT AND SHOWS SORT OF THE CHALLENGES THAT THE FIELD ITSELF IS TRYING TO DEAL WITH.

DR. CANET-AVILES: THANK YOU, PAT. THIS IS A SUPER RELEVANT COMMENT THAT YOU ARE MAKING AND SUGGESTION. AS I MENTIONED, LATER ON THIS IS ONE OF THREE APPROACHES THAT WE ARE TAKING. THE OTHER TWO APPROACHES, ONE OF THEM IS AN EXTERNAL DATA GATHERING. AND WE HAVE ENGAGED WITH A CONSULTANT COMPANY THAT IS HELPING US WITH EXACTLY WHAT YOU ARE TALKING ABOUT, A LANDSCAPE ANALYSIS AND KOL QUERY ABOUT WHAT ARE THE NEEDS AND THE BOTTLENECKS IN THE CLINICAL DEVELOPMENT OF MOST PREVALENT

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NEURODISEASES. BECAUSE WHEN WE GO FOR THE CLINICAL, WE OBVIOUSLY HAVE OUR RARE DISEASE PORTFOLIO. I THINK A VERY LARGE PERCENTAGE IS NEURO ALREADY. SO FOR RARE WE ARE KIND OF -- WE HAVE FOCUS ALREADY. FOR PREVALENT WE DON'T. THERE ARE PREVALENT DISEASES THAT ARE NEURO IN CALIFORNIA THAT ARE AFFECTING A VERY LARGE POPULATION THAT WE DO NOT HAVE AN ANALYSIS OF WHAT THE BOTTLENECKS IN THE PHARMA AND BIOTECH INDUSTRY ARE. AND THIS HAS BEEN DELEGATED TO AN EXTERNAL CONSULTANT THAT'S GATHERING THAT DATA.

SO WE WILL BE PRESENTING THIS AT THE AUGUST JOINT SCIENCE SUBCOMMITTEE/NEURO TASK FORCE BECAUSE THAT WILL HAVE TO DO WITH THE DATA FOR THE CELL AND GENE THERAPIES. AND WE WILL GO INTO A DEEP DIVE OF THAT. I HOPE THAT ANSWERS YOUR QUESTION, PAT.

DR. LEVITT: YEAH. I THINK THAT'S GREAT. I THINK GETTING SOME MAJOR INSIGHT THERE I THINK IS GOING TO BE REALLY IMPORTANT. I THINK MANY OF US WHO ARE ON THE NEURO TASK FORCE KIND OF KNOW THIS ANYWAY FROM OUR OWN EXPERIENCES AND OUR COLLEAGUES. BUT HAVING SOME INSIGHT INTO HOW WE CAN GET THROUGH THOSE ROADBLOCKS, WHICH IS NOT A -- IT'S NOT A COMMENTARY ON CIRM, IT'S NOT A COMMENTARY ON BIOTECH

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AND PHARMA. IT'S A COMMENTARY ON THE CHALLENGES OF ADDRESSING NEURO-RELATED DISEASES AND DISORDERS. I JUST WANT TO MAKE THAT CLEAR, THAT IT'S NOT -- THIS IS NOT A NEGATIVE COMMENTARY. THIS IS THE STATE OF THE FIELD.

DR. CANET-AVILES: THANK YOU, PAT. YOU ARE VERY INSIGHTFUL. ABSOLUTELY.

CHAIRMAN FISCHER-COLBRIE: GREAT. STEVE.

MR. JUELSGAARD: YES. SO THREE THINGS. THE FIRST IS LET ME APOLOGIZE FOR BEING LATE. I HAD THE START TIME OF THE MEETING WRONG. I THOUGHT IT WAS ONE INSTEAD OF 12. SO JUST A QUICK QUESTION.

THE SLIDES THAT HAVE BEEN PRESENTED, ARE THEY THE SAME SLIDES THAT ARE LISTED ON THE LINK FOR THE AGENDA FOR TODAY?

DR. CANET-AVILES: YES, THEY ARE, STEVE.

MR. JUELSGAARD: GREAT. SO I'M NOT MISSING ANYTHING.

SO ONE QUESTION FOR MY COLLEAGUES ON THE NEURO TASK FORCE, BECAUSE WE NEVER GOT THERE, AND I THOUGHT THAT MIGHT HAVE BEEN ONE OF OUR REMITS. AND THAT'S TO COME UP FROM THE NEURO TASK FORCE POINT OF VIEW WITH A GOAL AS TO WHAT WE THOUGHT SHOULD BE DONE, AND WE HAVEN'T DONE THAT. I MEAN THERE'S OBVIOUSLY BEEN A BIG SURVEY DONE OF A BUNCH OF



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PEOPLE, WHICH IS FINE, AND WE HAVE THAT DATA, WHICH IS FINE. BUT THE QUESTION IS SHOULD THE GROUP THAT WAS KIND OF TASKED WITH, I THINK ANYWAY, RUNNING TO THE GROUND WHAT IT IS THAT WE MIGHT BE MORE FOCUSED ON OR DOING JUST HASN'T REALLY HAPPENED. SO FOR ME THAT'S A BIT OF HOLE YET THAT HASN'T BEEN FILLED. MAYBE IT'S NEVER GOING TO GET FILLED. I DON'T KNOW. BUT I JUST WANT TO POINT THAT OUT.

THE THIRD THING, I WANT TO ADDRESS WHAT PAT SAID. SO LET ME ASK A QUESTION, ROSA. I'M GOING TO ASK ABOUT INDUSTRY RESPONSES BECAUSE YOU HAVE IN YOUR SLIDES, YOU STARTED WITH 679 NEUROSCIENTISTS THAT ARE ASKED ABOUT IT, AND YOU GOT 111 RESPONSES. SO ROUGHLY ONE IN SIX RESPONDED, IF THE MATH WORKS THAT WAY, I'M ASSUMING IT DOES, OR SOMEWHERE AROUND THERE. SO OBVIOUSLY WE HAVE A MINORITY OF RESPONDENTS.

BUT WHEN YOU LOOK AT INDUSTRY RESPONSE, MY BIG QUESTION IS DID YOU GET ANY RESPONSES FROM THE LARGE COMPANIES, FROM THE PFIZERS AND MERCKS AND SANOFIS AND NOVOS AND GENENTECHS, ROCHE, ALL OF THOSE COMPANIES WHO LIKELY ARE INVOLVED IN SOME OF THESE AREAS? BUT I'M WONDERING IF THEY MAY HAVE JUST DECIDED THEY'RE NOT GOING TO PARTICIPATE BECAUSE THEY REALLY DON'T SEE A ROLE FOR THEMSELVES

TO PLAY WITH CIRM.

CAN YOU HELP ME WITH WHO WERE THE KIND OF COMPANIES THAT WERE RESPONDING IN THE INDUSTRY RESPONSES?

DR. CANET-AVILES: WE ARE LOOKING INTO THIS. CAN YOU GIVE ME --

MR. JUELSGAARD: YEAH. IT'S JUST AN OVERVIEWING QUESTION. I THINK THIS IS TO PAT'S POINT. WHY DO WE NEED TO HAVE AN INTERSECTION BETWEEN CIRM AND THE BIOPHARMA INDUSTRY OTHER THAN WHAT WE HAVE NOW? YES, I AGREE WITH PAT. I THINK SOME OF THESE ARE REALLY DIFFICULT DISEASES, AND PEOPLE MAY SHY AWAY FROM THEM BECAUSE THEY ARE. BUT ON THE OTHER HAND, I DO THINK THAT THERE BIOPHARMA COMPANIES THAT ARE WORKING IN SOME OF THESE AREAS BUT JUST WE DON'T CONNECT WITH THEM. MAYBE WE CAN'T OR NEVER WILL BE ABLE TO CONNECT WITH THEM. BUT ONE OF THE QUESTIONS IS IS THERE SOMETHING MORE WE CAN DO BECAUSE ULTIMATELY THE COMMERCIALIZATION OF THESE THERAPIES OF ANY RESULT ARE LARGELY GOING TO BE DONE THROUGH INDUSTRY. THEY'RE THE ONES WHO ARE ABLE TO GET THINGS APPROVED BY THE FDA AND THEN OUT ON THE ROAD AND BEFORE PHYSICIANS TO UNDERSTAND WHAT THEY ARE AND THEN ULTIMATELY PRESCRIBED AND TAKEN BY PATIENTS.

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SO JUST SOME COMMENTS THAT OCCUR TO ME.  
AND, AGAIN, I APOLOGIZE FOR BEING LATE TO THE PARTY  
HERE.

DR. CANET-AVILES: THESE ARE VERY GOOD  
COMMENTS. WE WILL GET BACK TO YOU WITH THE IDENTITY  
OF THE ORGANIZATIONS THAT RESPONDED. BUT AS A QUICK  
LOOK, NONE OF THEM -- I DIDN'T SEE -- THEY WERE --  
NONE OF THEM WAS FROM CALIFORNIA. I CAN TELL YOU  
THAT FROM WHAT I SAW. THEY DON'T HAVE THE ALECTORS,  
THE DENALIS, THE GENENTECHS. SO WE WILL BE HAVE TO  
PROBABLY THINK ABOUT HOW TO APPROACH THAT GAP.

CHAIRMAN FISCHER-COLBRIE: SO PAT.

DR. LEVITT: I THINK FRED WAS FIRST.

CHAIRMAN FISCHER-COLBRIE: FRED, SORRY  
ABOUT THAT. I'M SORRY, ROSA. I CUT YOU OFF.

DR. CANET-AVILES: GO AHEAD. IT'S OKAY.

DR. FISHER: SURE. LOWER HAND.

TWO THINGS JUST TO RESPOND TO STEVE.  
STEVE, I APPRECIATE YOU POINTING OUT THAT THE NEURO  
TASK FORCE HAS YET TO GET TO SOME KIND OF DISCUSSION  
OF PRIORITIZATION OR EVEN APPROACH TO MAKING  
RECOMMENDATIONS. AND CERTAINLY SOME OF THE  
INFORMATION HERE MAY BE HELPFUL IN THAT REGARD. BUT  
I APPRECIATE YOU POINTING IT OUT, THAT WE STILL HAVE  
SIGNIFICANT WORK TO DO TO INFORM THIS COMMITTEE AND

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THE STRATEGIC ALLOCATION COMMITTEE ABOUT WHERE DOLLARS SHOULD GO.

THE OTHER THING IN TERMS OF INDUSTRY, AFTER SITTING THROUGH DOZENS AND DOZENS OF TRAN1 AND 2 AND CLIN1 AND 2, THE PROJECTS THAT ARE BEING FUNDED ARE SO FAR AWAY FROM THE STAGE WHERE INDUSTRY GETS INVOLVED, IT DOESN'T SURPRISE ME THAT INDUSTRY IS NOT WELL REPRESENTED BECAUSE THE THINGS WE'RE FUNDING, WHILE BEING IMPORTANT, THEY'RE JUST NOT AT THE STAGE WHERE INDUSTRY GETS INVOLVED.

AND WHEN IT COMES TO THE BIG NEURODEGENERATIVE DISEASES, THE TRACK RECORD OF FAILURE FOR PHASE 3 CLINICAL TRIALS CERTAINLY HAS COMPANIES CONCERNED. NOW, A FEW TAKE THE DEEP DIVE IN AND MOST ARE UNSUCCESSFUL. SO I THINK THE COMBINATION OF THE DISEASES WE'RE TRYING TO TACKLE BEING REALLY TOUGH AND THE FACT THAT THE PROJECTS WE'RE FUNDING ARE REALLY SO FAR AWAY FROM ATTRACTING INDUSTRY TO MOVE THEM FORWARD MIGHT HAVE SOMETHING TO DO WITH WHY WE DON'T HAVE A BETTER RESPONSE RATE FROM INDUSTRY.

DR. THOMAS: FRED, IF I CAN RESPOND AND ADD TO THAT. ONE OF THE ISSUES IS THAT IF YOU LOOK SORT OF HISTORICALLY OVER WHO'S APPLYING FOR GRANTS, IT ISN'T BIG PHARMA. AND AS A RESULT, THE

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RELATIONSHIPS THAT WE HAVE WOULD NOT SORT OF CATER TO GETTING RESPONSES FROM THEM ON THESE SORTS OF QUESTIONS. SO WE CAN TRY TO FIGURE OUT HOW TO DO A BETTER JOB OF GETTING SOME RESPONSES, BUT THESE ARE NOT, AS YOU SAY -- WHERE WE OPERATE IS NOT AT THE END OF THE RESEARCH SPECTRUM WHERE BIG PHARMA WOULD BE GETTING INVOLVED.

SO, STEVE, I'M NOT SURE WHAT THEIR ANSWERS WOULD BE TO THESE QUESTIONS. BUT WE CERTAINLY CAN TRY TO FIGURE OUT HOW TO GENERATE A RESPONSE FROM THAT COMMUNITY BECAUSE IT WOULD BE HELPFUL.

DR. FISHER: I THINK MECHANISMS OF ACTION AND BIOMARKERS WOULD CERTAINLY BE TOP OF MIND FOR INDUSTRY BECAUSE IT GIVES THEM A TARGET TO AIM FOR. WITHOUT THOSE THINGS, THERE REALLY IS NO TARGET, AND THEY'RE SORT OF SHOOTING BLIND.

DR. CANET-AVILES: I AGREE, FRED. SOMETHING THAT I MIGHT ADD IS WHEN I WAS LEADING THE PUBLIC/PRIVATE PARTNERSHIPS AT THE FOUNDATION FOR THE NATIONAL INSTITUTES OF HEALTH, WE HAD DIFFERENT PARTNERSHIPS AND DIFFERENT NEURODISEASE FOCUS WITH THE FDA PUBLIC PARTNERS AND NIH, BUT ALSO INDUSTRY. THE PFIZERS, THE LILYS, THE GENENTECHS, ALL THE NEURO-RELEVANT COMPANIES. AND WHAT THEY NEEDED WAS INVESTMENT IN EARLY DISCOVERY OF MECHANISMS FOR

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NOVEL TARGETS. SO WITH ALZHEIMER'S, WE'VE DEPLETED THE A BETA CASCADE. WE NEED NEW TARGETS THAT ARE COMING FROM THE NEURO-IMMUNE SYSTEM. AND THE OTHER ONE WAS BIOMARKERS. WE HAVE A LOT OF FAILURE IN THE CLINICAL TRIALS BECAUSE WE ARE NOT ABLE TO STRATIFY THE POPULATIONS PROPERLY. SO THOSE ARE SOME.

AND THEN DELIVERY OF THESE THERAPIES TO THE RIGHT POPULATIONS, RIGHT. SO THOSE -- AND WE ALL AS SCIENTISTS PARTICIPATE IN MEETINGS WHERE WE HAVE STAKEHOLDERS FROM INDUSTRY AS WELL AS ACADEMIA. AND THESE ARE ALWAYS THE TYPE OF ISSUES THAT ARE COMING TO THE TABLE.

THE APPROACH THAT WE WERE TAKING IN TERMS OF CLINICAL DEVELOPMENT, WE WERE GOING TO -- WE ARE LOOKING AT THE LANDSCAPE IN CALIFORNIA IN TERMS OF INVESTMENT IN CELL AND GENE THERAPIES AND ALL OVER THE WORLD AS WELL. AND THEN WHAT IS THE AMENABILITY AND WHAT ARE THE BOTTLENECKS PRECLUDING THAT FROM HAPPENING?

DR. FISHER: THANK YOU. SOUNDS EXACTLY RIGHT. THANK YOU.

CHAIRMAN FISCHER-COLBRIE: PAT.

DR. MELMED: VITO, SORRY. THIS WAS SCHEDULED TO END AT ONE. I HAVE TO LEAVE.

CHAIRMAN IMBASCIANI: OKAY. SHLOMO, THANK

YOU VERY MUCH.

DR. LEVITT: SO THERE ARE -- IN MY CONVERSATIONS WITH LITTLE PHARMA, BIG PHARMA, BIOTECH, WHATEVER, AND HAVING BEEN DOING THIS FOR A VERY LONG TIME, THERE ARE THREE MAJOR COMPONENTS THAT WE FACE, TWO OF WHICH WE HAVE NO CONTROL OVER. THE THIRD ONE WE DO. SO I'M THINKING -- THIS IS JUST IN TERMS OF PUTTING MY CIRM HAT ON, NOT FOR THE ENTIRE FIELD. ONE IS COMPLEXITY. WE CAN'T, CIRM CAN'T SIMPLIFY THE DISEASES AND DISORDERS. THEY ARE WHAT THEY ARE. HETEROGENEITY, WHICH SPEAKS TO THIS ISSUE ABOUT STRATIFICATION, ET CETERA, WE CAN'T CHANGE THAT. THESE ARE TWO FACTS, COMPLEXITY AND HETEROGENEITY.

THE THIRD ONE, WHICH COMES UP A LOT IN CONVERSATION AND IN OPINION PIECES AND REVIEWS, ET CETERA, IS REPRODUCIBILITY. AND NEURO FIELD ITSELF, REPRODUCIBILITY IS A REAL CHALLENGE. AND I THINK -- I RAISE THIS BECAUSE I THINK IN THE WAY THAT CIRM STRUCTURES THE REQUESTS FOR APPLICATIONS AND THE KINDS OF THINGS THAT CIRM INSISTS UPON, IF THEY COULD ADDRESS THIS ISSUE AROUND REPRODUCIBILITY, AND THERE ARE MANY WAYS OF DOING THAT -- I'M NOT GOING TO BRING IT UP TODAY -- INDUSTRY WOULD HAVE A MORE INTERESTED EYE TOWARDS WHAT THOSE WHO ARE SUPPORTED

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BY CIRM ARE DOING. IT IS REALLY DEMONSTRATING REPRODUCIBILITY.

A LOT OF THE FAILURES THAT HAVE OCCURRED ARE BECAUSE THERE'S A VERY HIGH PERCENTAGE OF MODEL SYSTEMS THAT CAN'T BE REPRODUCED ONCE THEY'RE BROUGHT INTO AN INDUSTRY PLATFORM FROM ACADEMICS. AND IT'S NOT ABOUT -- IT'S NOT ABOUT NEFARIOUS THINGS THAT ARE GOING ON. IT'S ABOUT WHAT NIH MAY NOT DO SO WELL, IN MY OPINION, BUT WHAT CIRM CAN DO WELL, WHICH IS TO ESTABLISH A FRAMEWORK IN WHICH REPRODUCIBILITY CAN ABSOLUTELY BE DEMONSTRATED, WHICH BRINGS A MUCH GREATER CONFIDENCE IN THE TARGETS DISCOVERY THAT WAS MENTIONED BY FRED AND BY OTHERS AND SO ON AND SO FORTH.

SO I THINK THERE IS ONE COMPONENT THAT WE CAN SPEAK TO IN FUTURE MEETINGS, WHICH IS THIS ISSUE. WE JUST CAN'T -- WE'RE NOT GOING TO SOLVE HETEROGENEITY AND COMPLEXITY, BUT WE CAN ADDRESS THIS. AND I THINK IT WOULD BE FAVORABLY VIEWED BY INDUSTRY. AND MAYBE THE CONSULTANT CAN GET SOME SENSE ABOUT WHETHER I'M ON TARGET OR NOT IN TERMS OF HOW ADDRESSING THAT WOULD BE VERY HELPFUL IN TERMS OF ENHANCING THE PARTNERSHIPS. THAT'S IT.

CHAIRMAN FISCHER-COLBRIE: PAT, I'VE ACTUALLY WRITTEN OPINION PIECES ON THIS BECAUSE THE



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PUBLISHED DATA INDICATE REPRODUCIBILITY RATES ARE RUNNING 25 PERCENT OR LOWER RELATED TO ACADEMIC SETTINGS. AND, HENCE, THE DECISION PROBLEMS LARGE PHARMA HAS TO WHICH PROGRAMS TO MOVE FORWARD. SO THIS IS A PARTICULAR HOT BUTTON OF MINE, I CAN GUARANTEE YOU; BUT, NONETHELESS, IT'S VERY IMPORTANT CONSIDERATIONS. AND I THINK THAT YOU'RE BRINGING UP AN EXCELLENT POINT FOR HOW IS IT THAT WE CAN DO THE BEST WE CAN TO WORK TOWARDS THE REPRODUCIBILITY QUOTIENT.

BY AND LARGE, NONE OF THAT IS NEFARIOUS. IT HAS TO DO WITH LAB PRACTICE, CONTROLS, PEOPLE WITH DIFFERENT RECIPES AND DIFFERENT EQUIPMENT, AND LACK OF RECORDING OF DATA IN PROPER CONSIDERATIONS AND FORMATS. AND IT'S REALLY AN OUTGROWTH OF A LOT OF WORK BEING IN A WAY PARALLEL TO PREINDUSTRIAL REVOLUTION OF AN ARTISAN MAKING A CHAIR, THEN HAVING A HARD TIME MAKING THE CHAIR THE NEXT TIME TYPE OF APPROACH. ANYWAY, ENOUGH.

DR. LEVITT: WHAT A METAPHOR. THAT WAS GREAT. THAT WAS A GREAT METAPHOR.

CHAIRMAN FISCHER-COLBRIE: WE'VE GOT TO GET INTO THE INDUSTRIAL REVOLUTION ON THAT. THAT DOES FRAME OUR ALPHA CLINIC -- NOT ALPHA CLINIC, BUT OUR FACILITIES FRAMEWORK QUESTION THOUGH BECAUSE TO

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INCREASE AUTOMATION, COMMONALITY OF DATASETS, AND, MOST IMPORTANT, THE COMMONALITY OF RECIPES ARE KEY PIECES TO THAT REPRODUCIBILITY QUOTIENT. AND THAT'S AN IMMEDIATE ROLE THAT CAN COME INTO PLAY RELATED TO OUR FACILITIES FUNDING THAT'S UNDER CONSIDERATION.

DR. LEVITT: MARK, THERE ARE WAYS, FOR EXAMPLE, OF INTRODUCING IN MODEL SYSTEMS GENETIC VARIATION WHICH IS SO IMPORTANT IN TERMS OF WHAT WE'RE TALKING ABOUT HERE. AND THERE ARE WAYS OF DOING IT. IT TAKES A LITTLE BIT MORE -- IT TAKES MORE TIME AND ENERGY TO DO IT, BUT IT'S NOT AS IF THAT CAN'T BE DONE. AND I THINK THAT'S PART OF THE CHALLENGE AS WELL, TO UNDERSTAND WHAT WE NEED TO INTRODUCE IN THE MODEL SYSTEMS THAT WILL GET US TOWARDS MECHANISM MUCH FASTER. AND ALL THESE THINGS WILL REFLECT WELL ON WHAT CIRM IS DOING, AND IT WILL RAISE THE INTEREST IN AT LEAST MORE CONVERSATIONS GOING ON WITH THE PRIVATE SECTOR, WHICH I THINK SO CRITICALLY IMPORTANT. ANYWAY, IT'S GOOD TO HEAR. I'M NOW GOING TO HAVE TO DIG AND FIND THAT EDITORIAL.

CHAIRMAN FISCHER-COLBRIE: DON'T WORRY. I HAVE A LOT OF OPINIONS ON THAT. GREAT COMMENTS. ALSO WANT TO ENSURE THAT IF THERE ARE COMMENTS OR QUESTIONS AROUND SORT OF THE WORKING GOALS OR

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HYPOTHESES THAT ARE BEING GENERATED, IF THERE'S ANY INITIAL REACTION TO THOSE ELEMENTS. AND OBVIOUSLY THERE'S AN INABILITY TO SPEND MUCH TIME THINKING ABOUT THAT, BUT WANTED TO DRAW OUT, IF THERE'S ANY INITIAL COMMENTARY, ON KIND OF WHAT YOU SEE AS TO WHERE THINGS ARE HEADED FROM PEOPLE HERE ON THE CALL. FRED.

DR. FISHER: YOU CAN ALWAYS COUNT ON ME TO HAVE SOMETHING TO SAY. I WAITED LONG ENOUGH FOR SOMEONE ELSE TO GO.

SO I THOUGHT IN THE CONTEXT OF THE WORK OF THE NEURO TASK FORCE, I APPRECIATED SORT OF THE HIGH LEVEL RESULTS, THAT THEY WERE FOCUSED ON SORT OF DISEASE MECHANISMS AND BIOMARKER RATHER THAN FOCUSED ON SPECIFIC DISEASES. THE NEURO TASK FORCE, AT LEAST IN MY OWN MIND, IT'S KIND OF A STRUGGLE BETWEEN ARE WE LOOKING TO CREATE EXPECTATIONS AROUND PERCENTAGE OF MONEY GOING TO SPECIFIC DISEASES OR ARE WE LOOKING TO TARGET FUNDING TOWARD UNDERSTANDING WHAT'S GOING ON WITH NEURODEGENERATIVE DISEASES?

SO I FOUND THAT AT LEAST INITIAL GOAL CONNECTED TO SORT OF DISEASES AND MECHANISMS OF ACTION, THE FACT THAT IT REALLY FRAMED IT AS SOMETHING MORE GENERAL RATHER THAN SOMETHING

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SPECIFIC TO SPECIFIC DISEASES. WE MIGHT END UP TARGETING SPECIFIC DISEASES AS A TASK FORCE. I DON'T KNOW. BUT I THINK THIS IS A SORT OF HELPFUL START TO JUMP INTO THAT HOLE THAT STEVE DESCRIBED.

CHAIRMAN FISCHER-COLBRIE: GREAT. I ALSO FIND IT QUITE TANTALIZING THAT THERE ARE SOME EARLY HINTS THAT THERE ARE CERTAIN MECHANISMS THAT MAY CONTRIBUTE TO MORE THAN ONE DISEASE CONDITION, FIRST OF ALL. AND SECOND, THAT THE EXPLORATION OF MORE NOVEL AREAS AROUND THE NEURO-IMMUNE SYSTEM AND THE GUT/BRAIN AXIS WILL ALL CONTRIBUTE TO BASE KNOWLEDGE THAT WILL HAVE FURTHER IMPACT AS WELL.

DR. FISHER: STEVE FINKBEINER AT GLADSTONE, WHO'S DONE A LOT OF WORK IN THIS AREA, HAS SORT OF A -- THE WAY HE DESCRIBES IT IS COMMON THREADS AMONG THE DIFFERENT NEURODEGENERATIVE DISEASES THAT HE'S IDENTIFIED. AND SO I THINK THERE IS WORK GOING ON AROUND THAT. AND IF WE WANTED TO PURSUE MORE OF THAT, THERE'S CERTAINLY PEOPLE IN THE FIELD THAT I THINK WOULD EMBRACE IT.

CHAIRMAN IMBASCIANI: SAY THAT NAME AGAIN PLEASE.

DR. FISHER: STEVE FINKBEINER AT GLADSTONE.

DR. CANET-AVILES: MAY I SAY SOMETHING?

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CHAIRMAN FISCHER-COLBRIE: SURE, ROSA.

DR. CANET-AVILES: THIS, OF COURSE, IS PRELIMINARY RECOMMENDATIONS AND IMPACT GOALS. BUT ONE OF THE IDEAS IS THAT, AT LEAST FOR THE DISCOVERY, THE INVESTMENT, WE COULD MAXIMIZE IT IF WE HAVE A FOCUS AROUND, SAY, SYSTEMS AND WE GATHER DATA AND SAY IN THE NEURO-IMMUNE AXIS WE ARE GATHERING DATA FROM DIFFERENT DISEASES, BUT IN A COMMON SYSTEM, THEN WE CAN ELUCIDATE COMMON TARGETS, COMMON BIOMARKERS. SAY IN ALZHEIMER'S OR PARKINSON'S, THERE MIGHT BE A NEURO-IMMUNE MARKER THAT IS COMMON OR NEURO-INFLAMMATORY MARKER THAT'S COMMON WITH MAJOR DEPRESSIVE DISORDER. SO WE COULD BE GATHERING DATA FROM ALL THESE, AND WE COULD BE ABLE TO VALIDATE ACROSS ALL THESE DISORDERS.

SO THAT'S MORE THE APPROACH THAT WE ARE THINKING ABOUT. AND THIS IS ALSO ALIGNED WITH THE DATA INFRASTRUCTURE THAT WE ARE PUTTING TOGETHER. WE ARE ON OUR THIRD PHASE. WE HAVE NOT PRESENTED. WE CAN PRESENT LATER ON AS WELL AS PART ALSO OF THE OVERALL RECOMMENDATIONS, HOW THINGS COULD ALL FEED TOGETHER. BUT I THINK THERE COULD BE MUCH MORE FOCUS, EVEN IF IT'S DISEASE AGNOSTIC, USE OF THE MONEY THAT WE CAN FUND IN ORDER TO TACKLE SOME OF THESE NEEDS THAT THE FIELD HAS AND LEVERAGE DATA

FROM MANY DISEASES.

DR. FISHER: A LOT OF THAT IS GOING ON NOW. TP43 IS IMPLICATED IN A NUMBER OF NEURODEGENERATIVE DISEASE INDICATIONS. ALPHA SYNUCLEIN IS IMPLICATED IN ALS AND PARKINSON'S. SO THERE ARE EXAMPLES ALREADY THAT THE FIELD HAS IDENTIFIED AROUND THESE TARGETS, COMMON TARGETS AMONG DIFFERENT NEURODEGENERATIVE DISEASES.

DR. CANET-AVILES: AND THEN WITH THIS, WE COULD THEN BE TO ABLE TO -- THE NEXT LAYER ON ALL OF THIS IS COLLABORATIONS. THERE ARE MANY CONSORTIAS TACKLING INDIVIDUAL DISEASES, AND WE COULD FOCUS. WHEN WE KNOW WHAT QUESTIONS WE WANT TO ANSWER, WE COULD HAVE SPECIFIC COLLABORATIONS WITH SOME OF THESE CONSORTIA, BRINGING THEM INTO THE FOLD.

BUT I'M SORRY I DIDN'T REALIZE THAT STEVE JUELSGAARD HAD HIS HAND RAISED.

CHAIRMAN FISCHER-COLBRIE: SORRY. STEVE.

MR. JUELSGAARD: NO. IT'S ALL RIGHT, ROSA. FINISH WHAT YOU WERE SAYING.

DR. CANET-AVILES: NO. THAT'S IT. I DON'T WANT TO BUILD TOO MUCH EITHER.

MR. JUELSGAARD: SO WE HAVE JUST SPENT A FAIR AMOUNT OF TIME, WHICH I THINK WAS VERY USEFUL IN TALKING ABOUT TWO PROBLEMS AREAS. ONE IS

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MECHANISM OF ACTION AND THE OTHER ARE BIOMARKERS FOR DISEASE, WHICH ARE STUMBLING BLOCKS TO GETTING TO THERAPIES, IS UNDERSTANDING HOW A DISEASE WORKS AND HOW YOU IDENTIFY THOSE WITH THAT DISEASE THAT YOU'RE GOING TO TRY AND TREAT. AND SO WE HAVE THIS -- WE HAVE AN APPROACH RIGHT NOW WHERE, IN OUR APPLICATION APPROACH, SO WE SAY, OKAY, ANYTHING INVOLVED WITH DISCOVERY, BRING IT ON IN. ANYTHING WITH TRANSLATION, BRING IT ON IN. WE DON'T DIFFERENTIATE.

ONE THING WE MIGHT CONSIDER IS ACTUALLY REFINING, AND MAYBE WE DO THIS ONCE A YEAR OR TWICE A YEAR OR SOMETHING LIKE THAT, BUT ASKING SPECIFICALLY FOR APPLICATIONS WHICH RELATE TO MECHANISM OF ACTION OR BIOMARKERS. AND WE PAY PEOPLE TO LOOK AT THOSE THINGS, AND THEY'RE CARVED OUT, STANDALONE BY THEMSELVES SO THAT WHEN THE GWG LOOKS AT THEM, THEY'RE COMPARING ONE PROJECT AGAINST ANOTHER, PROBABLY NOT ALL IN THE SAME DISEASE AREA, DIFFERENT DISEASE AREAS, BUT GETTING TO SEE WHAT LOOKS WORTHWHILE TO FUND. AND MAKE, OF COURSE, THIS OPEN, NOT JUST TO ACADEMIA, BUT TO INDUSTRY AS WELL.

I CAN ASSURE YOU THAT THERE'S A LOT OF WORK THAT GOES ON IN INDUSTRY IN BOTH OF THESE AREAS BECAUSE, IN ORDER TO DEVELOP A SUCCESSFUL THERAPY,

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INDUSTRY NEEDS TO KNOW THESE THINGS. AND THEY DON'T RELY SOLELY UPON IT COMING FROM THE ACADEMIC ENVIRONMENT TO TRY AND FIGURE IT OUT. THERE'S A LOT OF INTERNAL WORK THAT GOES ON IN BOTH OF THESE AREAS IN TRYING TO BRING A NEW THERAPY FORWARD.

JUST A THOUGHT. IT JUST OCCURRED TO ME AS WE WERE TALKING ABOUT SOME WAY TO TRY AND MOVE THIS PART OF THE PROBLEM FORWARD A LITTLE BIT BETTER.

DR. CANET-AVILES: THAT'S A GREAT IDEA. THAT CAN BE PART OF THE RECOMMENDATIONS. THIS WILL BE SOMETHING THAT WE WILL BRING INTO WHEN WE TALK ABOUT GOAL 4 ON JULY 11TH. SO THAT WILL BE IN THE RECOMMENDATIONS. THANK YOU, STEVE.

MR. JUELSGAARD: SURE.

CHAIRMAN FISCHER-COLBRIE: GREAT. OTHER QUESTIONS? ANYTHING AROUND THE PROCESS, TIMING, GOALS? THERE'S A LOT HERE. OBVIOUSLY THERE'S MORE INFORMATION PENDING. WHAT'S BEEN BROUGHT HERE IS THE ABILITY TO GET PEOPLE CAUGHT UP WITH ALL THE ACTIVITIES. I WANT TO ENSURE THAT THERE'S AN OPPORTUNITY FOR ANY KIND OF DISCUSSION ON ANY PARTICULAR TOPIC THERE. SO ARE THERE OTHER COMMENTS OR THOUGHTS THAT PEOPLE MIGHT HAVE? OBVIOUSLY PEOPLE CAN DIGEST THIS AND THERE WILL BE FOLLOW-ON DISCUSSIONS GOING FORWARD. STEVE.



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MR. JUELSGAARD: THIS IS A QUESTION FOR VITO. SO, VITO, WE'VE LOST -- THIS RELATES TO SOMETHING FRED, I BROUGHT UP ORIGINALLY, AND THEN FRED SORT OF FOLLOWED UP ON IT. AND IT'S THE UNCOMPLETED WORK OF THE NEURO TASK FORCE. WE HAVE TWO DIRECTIONS WE CAN GO. WE CAN JUST SAY, OKAY, IT'S UNCOMPLETED AND WE'VE DONE AS MUCH AS WE'RE GOING TO DO AND CALL IT A DAY, OR WE CAN CONTINUE THE EFFORT. BUT IN ORDER TO CONTINUE THE EFFORT, LARRY IS NOW GONE, WE NEED SOMEBODY TO LEAD THAT EFFORT. WE DON'T HAVE A VICE CHAIR OF THAT TASK FORCE OR WHATEVER. BY THE WAY, I'M NOT VOLUNTEERING, JUST TO BE CLEAR. I'M RAISING THIS.

CHAIRMAN IMBASCIANI: LOUD AND CLEAR.  
LOUD AND CLEAR. I HEAR YOU.

MR. JUELSGAARD: OKAY, GOOD. BUT I WONDER IF WE DON'T NEED SORT OF SOMEBODY TO TAKE THE LEAD. WE'VE HEARD SOME ELOQUENT VOICES TODAY OTHERWISE. AND MAYBE ONE OF THOSE PEOPLE WOULD BE WILLING TO DO THIS.

CHAIRMAN IMBASCIANI: THANK YOU, STEVE, FOR BRINGING THIS UP. I WAS ASKED FOR SOME COMMENTS EARLY ON, AND I BEGGED OFF BECAUSE I WANTED TO HEAR THIS WHOLE DISCUSSION BEFORE BASICALLY RAISING THE SAME POINT YOU JUST DID.

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I HAVE A QUESTION FOR THE NEURO TASK FORCE, BUT ONE THAT THE SCIENCE COMMITTEE MEMBERS ARE EQUALLY POISED TO HELP ME ANSWER. HOW MUCH WORK IS STILL LEFT FOR THE NEURO TASK FORCE TO DO? WE JUST WENT THROUGH NINE MONTHS OF AN INTENSE POSTGRADUATE COURSE IN THE GENETICS OF NEURODEGENERATIVE DISEASES, WHICH I LIKED. AND I JUST IMAGINE TRYING TO REPLICATE THAT FOR OTHER SUBDIVISIONS. I'LL BE HERE BEYOND MY TERMS, IF YOU WILL. SO THERE'S THAT QUESTION.

AND THEN THE MORE MUNDANE QUESTION IS, SINCE IS THERE IS MORE WORK FOR THE NEURO TASK FORCE, I THINK I'M GOING TO HAVE TO FIGURE OUT A WAY TO INSINUATE INTO THE JUNE AGENDA THE NAMING OF A NEURO TASK FORCE CHAIR. SO I GUESS I'M GOING TO -- YOU FOLKS WILL BE THE FIRST ONES TO HEAR IT. I'LL ECHO WHAT STEVE JUST SAID. MAYBE SOMEONE AMONG THIS AUGUST GROUP WHO ARE SO KNOWLEDGEABLE IN THIS WOULD BE WILLING TO HELM THAT TASK FORCE KNOWING THAT IT IS NOT A JOB THAT WILL LAST IN PERPETUITY, THAT IT HAS A LIMITED LIFE SPAN. BECAUSE AS WE GET CLOSER TO SEPTEMBER, I SEE THE STREAMS THAT ROSA AND THE REST OF THE LEADERSHIP TEAM IS WORKING ON THE SAF AND THE NEURO TASK FORCE WORK COMING CLOSER AND CLOSER TOGETHER. THAT IS, ONE CAN'T, ESPECIALLY THE

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SAF WORK, CAN'T REALLY WORK INDEPENDENTLY OF THE EFFORT OF THE OTHER.

SO IF PEOPLE WOULD LIKE TO GIVE ME A CALL OR EMAIL ME IF THEY DON'T WANT TO SAY ANYTHING PRESENTLY.

DR. THOMAS: CAN I JUST MAKE A COMMENT HERE, VITO, NOT ON THE QUESTION OF WHO WOULD BE THE NEXT AUGUST CHAIR. BUT AS WE KNOW, OVER THE COURSE OF THE LAST FEW MONTHS, WE'VE BEEN CONTEMPLATING THE JOINING OF THE NEURO TASK FORCE INTO THE LARGER SCIENCE SUBCOMMITTEE EFFORT WITH RESPECT TO THE STRATEGIC ALLOCATION FRAMEWORK BECAUSE IT'S ALL PART AND PARCEL OF THE SAME THING. SO I THINK THAT GOING FORWARD HERE, THE OBJECT IS TO HAVE FRANK CONSIDERATION -- BY THE WAY, THERE'S CONSIDERABLE OVERLAP BETWEEN THE PEOPLE ON THE TWO ENTITIES, BUT TO HAVE THE BENEFIT OF BOTH GROUPS ADDRESSING THE ISSUES OF THE FRAMEWORK PARTICULARLY WITH RESPECT TO THE NEURO ASPECT.

WE DON'T WANT THE TWO TO DIVERGE. WE BROUGHT THEM TOGETHER NOW, AND THAT'S HOW WE WANT IT GOING FORWARD.

CHAIRMAN IMBASCIANI: THANKS, J.T., FOR THAT. FIRST OF ALL, WITH THE INCLUSION OF MARIA BONNEVILLE AND MYSELF, THERE ARE SEVEN MEMBERS OF

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THE BOARD WHO ARE ON BOTH OF THE COMMITTEES. AND I AGREE WITH YOU, NOT DIVERGENT, BUT CONVERGENT, WHICH I THINK WAS THE WORD I USED, BUT I'M HESITANT TO SAY THAT I WANT TO -- I DON'T WANT TO MERGE THE NEURO TASK FORCE BECAUSE I THINK THAT WOULD BE DOUBLING SOME PEOPLE'S WORKLOAD. AND OUR BOARD MEMBERS ARE ALREADY TASKED WITH A LOT OF WORK TO DO THEIR DUE DILIGENCE.

BUT THE IDEA OF HAVING JOINT MEETINGS FOR AS LONG AS IS NECESSARY BETWEEN THE NEURO TASK FORCE AND SCIENCE SOUNDS LIKE A VERY EFFICIENT, LOGICAL WAY TO GO FORWARD.

DR. THOMAS: YES. I THINK AS ROSA SET FORTH IN HER TIMELINE, THAT IS THE PLAN FROM NOW UNTIL SEPTEMBER.

CHAIRMAN IMBASCIANI: GREAT.

MR. JUELSGAARD: SO FRED HAS HIS HAND RAISED.

DR. FISHER: I THINK THAT WHAT WAS JUST DESCRIBED BY J.T. MAKES A LOT OF SENSE. I SEE THE THREE SORT OF EFFORTS THAT ARE UNDER WAY, STRATEGIC ALLOCATION, THE SCIENCE SUBCOMMITTEE, THE NEURO TASK FORCE, I THINK THAT THE NEURO TASK FORCE WOULD BENEFIT FROM THE THINKING OF THE SCIENCE SUBCOMMITTEE AS WE CONTEMPLATE DIFFERENT APPROACHES

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TO INFORM THE STRATEGIC ALLOCATION. SO I LIKE THE IDEA OF NOT DISSOLVING THE NEURO TASK FORCE BECAUSE I THINK THERE IS A LOT OF GOOD WORK STILL TO BE DONE, AND NOT MERGING THEM INTO ONE ENTITY, BUT HAVING JOINT MEETINGS OF THE TWO INFORMED BY THE GREAT STAFF THAT WE HAVE INVOLVED IN THIS MAKES A LOT OF SENSE.

CHAIRMAN FISCHER-COLBRIE: OKAY.

MR. JUELSGAARD: I'M IN AGREEMENT WITH THAT AS WELL. THE ONLY THING I WOULD POINT OUT IS THAT SO FAR THE WORK THAT'S BEEN DONE, I'M NOT BEING CRITICAL OF IT, BUT ROUGHLY ONE-THIRD, SO I'M LOOKING AT SLIDE 8, WE HAVE 3.86 BILLION TO SPEND AND 1.14 BILLION OF THAT IS IN THE NEURO AREA. SO 31 PERCENT, SOMETHING LIKE THAT. SO A SUBSTANTIAL AMOUNT OF THE MONEY IS SUPPOSED TO BE ALLOCATED TO THE NEURO AREA. BUT IN WHAT WE'RE LOOKING AT SO FAR, I DON'T SEE MUCH OF AN EMPHASIS ON THE NEURO AREA. SO I THINK WHAT WE NEED TO DO IS PIVOT A LITTLE BIT TO TRY TO INTEGRATE, AS I SAID, A LITTLE MORE EMPHASIS IN THE NEURO AREA WITHIN OUR PRIORITIZATION OR WHATEVER, THAT WE MAKE SURE WE'RE SPENDING THE MONEY THERE IN A WAY THAT WE NEED TO BE SPENDING IT.

AND MAYBE THAT GOES BACK TO A POINT I MADE

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EARLIER. MAYBE WHEN WE TALK ABOUT BIOMARKERS OR MECHANISMS OF ACTION, WE ASK ABOUT IT FOR NEURODEGENERATIVE DISEASES. I DON'T KNOW IF THEY APPLY SO MUCH TO NEURO INJURY, BUT THEY CERTAINLY DO, I THINK, TO NEURODEGENERATION AS PART OF THE EFFORT TO SPEND THE 1.14 IN THE REMAINING NEURO BUDGET. IN ANY EVENT, WE NEED TO THINK ABOUT THE NEURO BUDGET AS KIND OF THE SEPARATE AMOUNT OF MONEY THAT'S BEING SPENT, AND WE NEED TO HAVE KIND OF GOALS AND INSPIRATIONS AROUND IT EMBEDDED WITHIN THE GENERAL GOALS AND ASPIRATIONS.

DR. LEVITT: THAT'S REALLY IMPORTANT, RIGHT, BECAUSE IT'S GOING TO TAKE STRATEGIES THAT ARE OVERLAPPING, BUT NOT IDENTICAL. RIGHT? BECAUSE THERE IS THIS ALLOCATION, AND WE WANT TO BE LIKE OUTSTANDING STEWARDS. IT'S NOT JUST TO SPEND THE MONEY, BUT IT'S TO MAKE SURE THAT IT'S BEING PLANNED OUT AND DONE REALLY WISELY AND IN THE MOST COMPLICATED AREA THAT CIRM IS DEALING WITH IN TERMS OF PROMOTING RESEARCH AND BEING FORWARD THINKING ABOUT THIS.

SO THERE STILL'S NEEDS TO BE SOME FOCUS THERE BECAUSE I THINK SOME OF WHAT HAS TO BE DEVELOPED IS A PLAN AND APPROACHES THAT TAKE INTO ACCOUNT THE CHALLENGES THAT WE'VE DISCUSSED AND THAT

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WE ALL KNOW ABOUT IN TERMS OF THE NEURO AREA. AND WE DON'T WANT TO DETRACT FROM THE REST OF WHAT THE SCIENCE SUBCOMMITTEE HAS TO DEAL WITH, RIGHT, WHICH IS STILL THE MAJORITY OF THE PROGRAMS. THIRTY-THREE PERCENT IS A BIG CHUNK, BUT IT'S STILL NOT THE MAJORITY. SO WE HAVE TO THINK CAREFULLY ABOUT HOW TO DO THIS BECAUSE I THINK IT'S A REAL CHALLENGE. IT'S AN IMPORTANT CHALLENGE TO HAVE. STOP THERE.

DR. THOMAS: CAN I JUST RESPOND, MARK?

CHAIRMAN FISCHER-COLBRIE: J.T. YOU HAVE A COMMENT, J.T.?

DR. THOMAS: YES. JUST TO REFINE A BIT WHAT STEVE AND PAT JUST SAID. I THINK IF YOU WERE TO LOOK, STEVE, AT WHAT HAS BEEN PUT INTO NEURO SINCE THE PASSAGE OF PROP 14 AS A PERCENTAGE OF HOW MUCH CIRM HAS PUT OUT OVERALL, IT ACTUALLY IS SOMEWHERE AROUND 30 PERCENT, GIVE OR TAKE. 33 PERCENT. SO THERE HAS BEEN QUITE A BIT FOCUS IN NEURO.

NOW, WHAT HASN'T BEEN DONE, WHICH IS THE PURPOSE OF THE STRATEGIC ALLOCATION FRAMEWORK EXERCISE, IS TO REFINE THAT THINKING IN THE NEURO SPACE AS TO WHERE YOU WANT TO FOCUS ON. AND THAT'S ALL A VERY IMPORTANT ELEMENT OF THE STRATEGIC UNDERTAKING WE'RE IN THE MIDDLE OF.

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CHAIRMAN FISCHER-COLBRIE: FRED.

DR. FISHER: SO WHEN I THINK ABOUT THE MISSION OF CIRM AND KIND OF WHAT WAS MARKETED AS PART OF ITS PASSAGE WAS CURES AND CREATING CURES FOR CALIFORNIANS. AND IT OCCURS TO ME THAT THE RESEARCH THAT WE ARE FUNDING IS A LONG WAY FROM PRODUCING CURES. AND I WORRY THAT WE WON'T HAVE ENOUGH EVIDENCE OR THE VOTERS WHEN WE FOCUS ON BASIC SCIENCE BECAUSE THAT MEANS IT'S LIKE TEN YEARS IF IT GETS THROUGH A PHASE 3 CLINICAL TRIAL AND FDA APPROVAL. SO I JUST WANT TO PUT THAT OUT THERE IN TERMS OF WHAT THE ALLOCATION OF THE FUNDING IS FOR AND HOW IT GETS US TO HAVING EVIDENCE OF SUCCESS THAT THE VOTERS WILL APPRECIATE DOWN THE ROAD. AND IT'S NOT TOO EARLY TO BE THINKING ABOUT THAT. PARTICULARLY AS WE RETHINK HOW WE'RE SPENDING MONEY, I JUST WANTED TO KIND OF STICK THAT IN THERE AS PART OF ONE OF THE PROBLEMS MAYBE.

DR. CANET-AVILES: FRED OR MARK, CAN I MAKE A COMMENT? VITO, CAN I MAKE A COMMENT?

CHAIRMAN IMBASCIANI: ABSOLUTELY.

DR. CANET-AVILES: YOU JUST HIT THE POINT, THE MAIN POINT, FRED, WHERE THE LEADERSHIP TEAM STARTED FROM, WHICH WAS WHERE WILL CIRM MAKE ITS BIGGEST IMPACT IN ITS LIFETIME. AND IT CANNOT JUST



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BE CELL AND GENE THERAPY APPROVALS, ESPECIALLY BECAUSE IN SOME CASES WE ALSO HAVE EDUCATIONAL, INFRASTRUCTURE, AND ACCESSIBILITY OF THOSE THERAPIES. WE HAVE TO FIGURE OUT ALSO THAT IN SOME CASES THE FIELD IS NOT READY. NEURO FIELD IS 30 YEARS BEHIND THE ONCOLOGY FIELD. WE HAVE TO LEARN LESSONS FROM OTHER DISEASES IN ORDER TO ADVANCE IT. AND WE HAVE A MANDATE THAT MAKES US USE STEM CELLS AND GENETIC RESEARCH AND THERAPIES. WE CAN USE THAT. WE CAN LEVERAGE IT TO ANSWER SOME QUESTIONS THAT NOBODY ELSE IS GOING TO ANSWER BECAUSE OF EITHER INDUSTRY IS NOT INVESTED IN THE EARLIER TRANSLATION OF THESE THERAPIES. THEY JUST NEED THOSE TOOLS.

SO, FOR EXAMPLE, RIGHT NOW WE HAVE FAILURES OF TRIALS IN NEURO AND AUTISM SPECTRUM DISORDERS THAT, IF THEY HAD A BIOMARKER TO HELP THEM STRATIFY THEM EARLIER ON, COULD MAKE IT SUCCESSFUL. SO IF WE HAVE AT CIRM THE STAMP THAT CIRM HAS MADE -- HELPED CATALYZE THE IDENTIFICATION AND VALIDATION OF X TARGET OR X BIOMARKER, THAT WILL ENSURE THE SUCCESS IN THIS DISEASE THAT IS PREVALENT. THAT IS ONE OF THE IMPACT GOALS THAT WE ARE ADDING TO THE IMPACT GOALS THAT ARE IN DRAFT RIGHT NOW. SO IT'S NOT ONLY IN CELL/GENE THERAPY

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APPROVALS. WE ARE ALSO TAKING INTO ACCOUNT THAT THE FIELD IN SOME DISEASES NEEDS HELP AT EARLIER STAGES. AND THAT IS SOMETHING THAT'S VERY IMPORTANT TO REALIZE. WE NEED TO SHIFT OUR FOCUS AND THE WAY THAT WE ARE THINKING BECAUSE WE HAVE AN OPPORTUNITY HERE THAT'S UNIQUE.

NOW, HOW DO WE MAXIMIZE THIS IS BY LEVERAGING THE TOOLS THAT WE ARE PUTTING TO GO ACROSS DISEASES. AND THE FIELD IS ALSO READY TO DO THAT. SO I'LL STOP HERE, BUT THAT'S HOW WE ARE THINKING ABOUT IT.

DR. THOMAS: FRED, I WOULD JUST ADD TO THAT THAT OBVIOUSLY THIS IS JUST ONE COMPONENT OF THE OVERALL STRATEGY THAT WE'RE PUTTING TOGETHER. SO WE WILL HAVE OUR RECOMMENDATIONS WITH RESPECT TO ISSUES ALL ALONG THE RESEARCH SPECTRUM, NOT JUST THE BASIC. BUT I THINK ROSA RAISES A VERY IMPORTANT POINT, WHICH IS, WITH RESPECT TO A LOT OF THE NEUROLOGICAL DISORDERS IN PARTICULAR, IF WE'RE SUCCESSFUL IN COMING UP WITH SOME FINDINGS THAT ENABLE FURTHER RESEARCH DOWN THE ROAD TO BE SUCCESSFUL, THAT'S A BIG DEAL IN THE FIELD BECAUSE THAT'S SOMETHING THAT'S NOT BEING DONE TO A LARGE EXTENT. BUT, AGAIN, WE WILL HAVE RECOMMENDATIONS ALONG THE SPECTRUM.

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DR. FISHER: THAT SOUNDS GREAT.

CHAIRMAN FISCHER-COLBRIE: GREAT. OTHER QUESTIONS OR COMMENTS? I'M NOT TRYING TO SHUT OFF THE MEETING OR ANYTHING BECAUSE WE DO HAVE ALLOCATED TIME. JUDY.

DR. GASSON: I THINK THIS HAS BEEN A GREAT DISCUSSION. AND THANK YOU, ROSA, AND EVERYBODY THAT'S PARTICIPATED. I JUST WANTED TO RAISE ONE OTHER CONSIDERATION THAT I THINK WE'VE TALKED ABOUT IN THE PAST. AND THAT IS WHAT SORTS OF RESOURCES FROM NIH AND FOUNDATIONS AND OTHER SORTS OF ORGANIZATIONS ARE GOING INTO STUDYING THESE DISEASES AND THE MODELS? AND HOW CAN WHAT CIRM DOES DISTINGUISH ITSELF FROM OTHER TYPES OF INVESTMENTS THAT ARE ONGOING AND POTENTIALLY BE COMPLEMENTARY TO OTHER TYPES OF INVESTMENTS THAT ARE GOING ON?

I JUST WONDERED IF THAT WAS SOMETHING THAT WE COULD TALK ABOUT AT A FUTURE TIME.

CHAIRMAN FISCHER-COLBRIE: GO AHEAD, ROSA.

DR. CANET-AVILES: CAN I ANSWER? SO THANK YOU, JUDY. THAT IS VERY RELEVANT. THAT WAS PART OF WHAT I WAS TRYING TO SAY IN TERMS OF THE LAYER THAT WILL COME LATER. SO FIRST OF ALL, WE ARE EVALUATING IN THIS DATA GATHERING, WE ARE EVALUATING WHAT IS THAT NIH AND OTHER ORGANIZATIONS ARE INVESTING IN

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THE DIFFERENT RESEARCH PHASES OF THESE DIFFERENT DISEASES. SO WE ARE GATHERING THAT DATA, AND THAT WILL BE PRESENTED AS WELL IN SOME WAY.

THE SECOND THING IS THAT WE ARE ALREADY CURRENTLY IN TOUCH AND IN DISCUSSIONS WITH NIH INSTITUTES TO LEVERAGE. SO NIMH FOR SURE BECAUSE OF REMIND. BUT I THINK WHAT'S IMPORTANT WHEN PEOPLE DEVELOP OR WHEN ORGANIZATIONS DEVELOP CONSORTIA AND COLLABORATIONS IS TO HAVE A FOCUS AND A VALUE PROPOSITION. WHAT IS IT THAT WE WANT TO DO TOGETHER? AND UP UNTIL WE ARE DEFINED WITH OUR RECOMMENDATIONS, IT'S NOT GOING TO BE A TIME FOR US TO START COLLABORATING. BUT WE HAVE THE RELATIONSHIPS. WE ARE READY TO GO. WE JUST NEED TO HAVE THIS DEFINITION, AND THAT'S ABSOLUTELY, BECAUSE WE NEED TO LEVERAGE EACH OTHER'S RESOURCES, AND WE HAVE AN ADVANTAGE WITH THE RELATIONSHIPS THAT WE HAVE ALREADY IN PLACE WITH THEM THROUGH ME AND THROUGH OUR COLLEAGUES IN THE LEADERSHIP TEAM.

DR. GASSON: THANK YOU, ROSA.

DR. CANET-AVILES: AND I WANT TO MAKE A SHOUT TO THE LEADERSHIP TEAM AS WELL BECAUSE THIS IS OBVIOUSLY J.T. AND I HERE AND SARA, BUT THERE'S A WHOLE LEADERSHIP TEAM THAT HAS BEEN WORKING VERY HARD IN THIS. SHYAM PATEL, ABLA CREASEY, GIL

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SAMBRANO, JEN LEWIS, RAFAEL AGUIRRE-SACASA, AND GEOFF LOMAX, I JUST WANT TO ACKNOWLEDGE THEM BECAUSE THEY ARE GREAT COLLEAGUES. WE ARE ALL WORKING VERY HARD ON THIS. AND I ALSO WANT TO ACKNOWLEDGE THOMAS TRIN. SORRY. JUST NEED TO SAY THE NAMES. AND THE WHOLE SCIENCE TEAM AT CIRM WHO'S BEEN ALSO DIGGING REALLY DEEPLY IN A LOT OF DATA. SO THANK YOU.

DR. THOMAS: CAN I ADD ONE OTHER THING, JUDY? SO THESE RELATIONSHIPS ARE VERY IMPORTANT AS WE DEVELOP THIS PLAN IN A NUMBER OF WAYS, WHICH ROSA REFERS TO, BUT AS AN EXAMPLE. SO WE ARE DEVELOPING OUR PLAN FOR HOW TO DEAL WITH RARE DISEASES AS PART OF THIS OVERALL PROJECT. AND ABLA THROUGH HER RELATIONSHIP WITH PETER MARKS AT THE FDA AND MANY OTHERS IS PUTTING TOGETHER A BLUE CHIP TEAM OF ADVISORS ON WHAT DIRECTION WE SHOULD BE GOING IN RARE DISEASE.

SO ALL OF THESE RELATIONSHIPS ARE VERY IMPORTANT AND WILL FACTOR INTO THE ULTIMATE SET OF RECOMMENDATIONS. JUST WANT EVERYBODY ON THIS GROUP TO KNOW THAT THESE RELATIONSHIPS ARE BEING BROUGHT TO THE FORE AS PART OF THIS EFFORT.

CHAIRMAN FISCHER-COLBRIE: VITO.

CHAIRMAN IMBASCIANI: THANKS. GOING TO BE MY FINAL COMMENT. I JUST WANT TO AMPLIFY JUDY'S

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THANK YOU TO THE TEAM THAT PUT THIS TOGETHER. AND THIS IS A PUBLIC MEETING, SO, ROSA, THANK YOU FOR NAMING ALL OF THE PEOPLE THAT DESERVE THANKS. I DON'T KNOW HOW YOU DO IT. YOU HIT SO MANY NAILS ON THE HEAD ALL AT ONE SWING. THIS PRESENTATION WAS SO SMOOTH, I THINK IT BELIES -- IT DOESN'T DEMONSTRATE THE SOPHISTICATION OF THE THINKING OF THE TEAM THAT WENT INTO THIS AND YOUR HIGH LEVEL DISCUSSIONS. I REALLY APPRECIATE THE WORK OF EVERYONE. AND, J.T., THANK YOU VERY MUCH FOR EMPOWERING THE LEADERSHIP TEAM TO DO ALL OF THIS GREAT WORK.

DR. THOMAS: THANK YOU, VITO.

CHAIRMAN FISCHER-COLBRIE: WELL SAID. WELL SAID. GREAT. NOTWITHSTANDING ANY OTHER LAST MINUTE COMMENTS, I THINK WE'RE ABOUT READY TO ADJOURN.

DR. THOMAS: WE HAVE A PUBLIC COMMENT HERE, I THINK.

CHAIRMAN FISCHER-COLBRIE: VERY IMPORTANT POINTS. I NEED TO BE TRAINED. ARE THERE PUBLIC COMMENTS?

MS. MANDAC: YES. WE DO HAVE ONE MEMBER OF THE PUBLIC WHO'S IN THE ROOM WITH US TODAY. YOU WILL HAVE THREE MINUTES. WE'LL HAVE A TIMER START AND IT WILL SHOW ON THE SCREEN.

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MS. KUNWAR: THANK YOU SO MUCH. I AM PRIVY KUNWAR WITH PRECISION NEUROMED, AND WE'RE A CNS THERAPEUTICS COMPANY. I'M VERY GRATEFUL FOR THE OPPORTUNITY TO ADDRESS BOTH THE JOINT SCIENCE SUBCOMMITTEE AND THE NEURO TASK FORCE.

PRECISION NEUROMED IS BUILDING A TARGETED DRUG DELIVERY PLATFORM FOR THERAPIES TO THE BRAIN WITH AN INITIAL FOCUS ON GLIOBLASTOMA. WE AIM TO BRING A PHASE 3 CLINICAL TRIAL FOR GLIOBLASTOMA TO FRUITION IN THE NEXT FEW YEARS.

WE HOPE THAT CIRM WILL CONTINUE TO PURSUE SCIENTIFIC AND TECHNICAL ADVANCES IN NEURODELIVERY SYSTEMS THAT AIM TO AGE EFFECTIVELY AND REPRODUCIBLY DELIVER TARGETED THERAPIES ACROSS THE BLOOD-BRAIN BARRIER.

PATIENTS WITH CNS CONDITIONS CAN RECEIVE THESE THERAPIES. AND WE DEFINITELY AIM TO APPLY FOR CIRM GRANTS. THANK YOU.

CHAIRMAN IMBASCIANI: THANK YOU.

DR. THOMAS: THANK YOU FOR COMING.

CHAIRMAN FISCHER-COLBRIE: GREAT. I THINK THAT CONCLUDES THE MEETING; IS THAT CORRECT?

MR. TOCHER: IT DOES.

CHAIRMAN FISCHER-COLBRIE: THANK YOU SO MUCH. I REALLY APPRECIATE EVERYONE'S PARTICIPATION

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ON THE CALL AND LOOKING FORWARD TO AN ONGOING  
PROCESS HERE TO BE ABLE TO SHAPE THE ACTIVITIES OF  
CIRM GOING FORWARD. AND, AGAIN, I'M SO IMPRESSED  
WITH THE TEAM, THE THINKING, THE WORK, THE  
LEADERSHIP ON THIS. SO I'M VERY MUCH EXCITED ABOUT  
SEEING WHAT THE NEXT STEPS ARE AND CONTINUING TO SEE  
HOW WE VOLUNTEERS CAN WORK TO SUPPORT CIRM'S EFFORT  
TO CONTINUE TO MAKE ADVANCES. SO THANK YOU VERY  
MUCH.

DR. THOMAS: THANK YOU.

DR. FISHER: THANK YOU. GREAT JOB, MARK.

(THE MEETING WAS THEN CONCLUDED AT 1:45 P.M.)



**REPORTER'S CERTIFICATE**

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE 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 JUNE 14, 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.

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