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

REGULAR MEETING

LOCATION: VIA ZOOM

DATE: MARCH 5, 2026
10 A.M.

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

FILE NO.: 2026-7

I N D E X

ITEM DESCRIPTION	PAGE NO.
OPEN SESSION	
1. CALL TO ORDER	3
2. ROLL CALL	3
3. DISCUSSION OF PROGRAM PREFERENCES AND GUIDING PRINCIPLES PDEV CONCEPT PLAN (PDEV) CLINICAL-2 CONCEPT PLAN (CLIN2) DISCOVERY-4 CONCEPT PLAN (DISC4)	8
CLOSED SESSION	**NONE**
4. DISCUSSION OF CONFIDENTIAL INTELLECTUAL PROPERTY OR WORK PRODUCT, PREPUBLICATION DATA, FINANCIAL INFORMATION, CONFIDENTIAL SCIENTIFIC RESEARCH OR DATA, AND OTHER PROPRIETARY INFORMATION RELATING TO THE CIRM PORTFOLIO. (HEALTH & SAFETY CODE 125290.30(F)(3)(B) AND (C)).	
OPEN SESSION	
5. PUBLIC COMMENT	NONE
6. ADJOURNMENT	104

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MARCH 5, 2026; 10 A.M.

CHAIRMAN FISCHER-COLBRIE: CALL THE SCIENCE SUBCOMMITTEE MEETING TO ORDER. AND, SCOTT, IF YOU COULD TAKE THE ROLL.

MR. TOCHER: SURE. MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: PRESENT.

MR. TOCHER: MONICA CARSON.

DR. CARSON: PRESENT.

MR. TOCHER: MARK FISCHER-COLBRIE.

CHAIRMAN FISCHER-COLBRIE: HERE.

MR. TOCHER: ELENA FLOWERS.

DR. FLOWERS: PRESENT.

MR. TOCHER: JUDY GASSON.

DR. GASSON: HERE.

MR. TOCHER: VITO IMBASCIANI.

CHAIRMAN IMBASCIANI: HERE.

MR. TOCHER: PAT LEVITT.

DR. LEVITT: HERE.

MR. TOCHER: SHLOMO MELMED. CAROLYN MELTZER.

DR. MELTZER: HERE.

MR. TOCHER: CHRIS MIASKOWSKI.

DR. MIASKOWSKI: PRESENT.

MR. TOCHER: SHAUNA STARK. KAROL WATSON.

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DR. WATSON: HERE.

MR. TOCHER: KEITH YAMAMOTO.

DR. YAMAMOTO: HERE.

MR. TOCHER: THANK YOU VERY MUCH. WE HAVE A QUORUM. MARK, BACK TO YOU.

CHAIRMAN FISCHER-COLBRIE: GREAT. AND, J.T., IF YOU'D LIKE TO KICK OFF WITH OPENING REMARKS.

DR. THOMAS: THANK YOU VERY MUCH, MARK. GOOD MORNING, MEMBERS OF THE SCIENCE SUBCOMMITTEE AND MEMBERS OF THE PUBLIC, AND TO OUR VERY HARDWORKING TEAM. RUN OF SHOW FOR TODAY'S PRESENTATION, I'M, AS MARK SUGGESTS, GOING TO GIVE SOME BRIEF OPENING REMARKS. WE'RE THEN GOING TO PROCEED WITH SHYAM AND GIL. SHYAM IS PINCH-HITTING FOR ROSA TODAY WHO'S CURRENTLY CO-CHAIRING A NATIONAL ACADEMIES CONFERENCE ON PRECISION MEDICINE AND NEUROSCIENCE IN WASHINGTON, D.C. AND SENDS HER BEST. BUT IN THE MEANTIME, SHYAM IS PINCH-HITTING. AND SO IT WILL BE SHYAM AND THEN GIL AND THEN BACK TO SHYAM FOR A WRAP-UP, AND THEN WE'LL BE HAVING DISCUSSION THROUGHOUT. SO THAT'S KIND OF THE ORDER OF THINGS.

SO JUST BY WAY OF INTRODUCTION, START OUT RECOGNIZING THAT EVERYTHING WE DO, OF COURSE,

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1 INCLUDING ALL THE PROCESSES WE'RE GOING TO BE
2 DISCUSSING TODAY FOR FUNDING MECHANISMS, ARE, AS
3 ALWAYS, IN THE CONSTANT QUEST TO GENERATE POTENTIAL
4 THERAPEUTIC TREATMENTS FOR OUR PATIENTS FOR
5 CURRENTLY UNCURABLE DISEASES.
6 THIS PROCESS BEGAN BACK IN LATE '23 WHEN
7 THE BOARD HAD A DISCUSSION ABOUT THE FACT THAT WE
8 HAD A FINITE AMOUNT OF MONEY REMAINING TO DEPLOY
9 GOING FORWARD FOR THE LIFE OF THE PROP 14 FUNDS AND
10 DIRECTED THE TEAM TO UNDERTAKE A PRIORITIZATION
11 PROCESS TO DETERMINE WHERE BEST TO SPEND THOSE
12 DOLLARS.
13 THAT CONVERSATION WAS FOLLOWED IN FAIRLY
14 SHORT ORDER BY A VERY DRAMATIC INCREASE IN
15 APPLICATIONS ACROSS THE BOARD OF OUR VARIOUS
16 PROGRAMS WHICH FURTHER EMPHASIZED THE URGENCY OF THE
17 PRIORITIZATION PROCESS.
18 SO AS YOU ALL WILL RECALL, WE THEN
19 IMPLEMENTED A PAUSE OVER A PERIOD OF MONTHS WHILE WE
20 UNDERTOOK AN INTENSIVE, DATA-DRIVEN ANALYSIS OF
21 WHERE BEST TO SPEND OUR FUNDS GOING FORWARD, WHICH
22 PROCESS ENDED UP IN SEPTEMBER OF '24 WITH THE
23 ADOPTION BY THE BOARD OF THE STRATEGIC ALLOCATION
24 FRAMEWORK OR SAF. THAT SAF INCLUDED A NUMBER OF
25 IMPACT GOALS TO ACHIEVE VARIOUS RESULTS, WHICH, IN

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1 TURN, HAD A SET OF GUIDING PRINCIPLES WHICH WERE
2 ADOPTED TO DIRECT FUNDING TOWARDS THE OPTIMAL GRANTS
3 AS THEY WOULD BE REVIEWED OVER TIME IN THE VARIOUS
4 PROGRAMS IN OUR PORTFOLIO.
5 TO OPERATIONALIZE THE GUIDING PRINCIPLES,
6 THE TEAM DEVELOPED AND PRESENTED A SET OF
7 PREFERENCES WHICH WERE DESIGNED TO EMPHASIZE CERTAIN
8 ELEMENTS OF WHAT WE WOULD BE LOOKING FOR IN OUR
9 PROGRAMS GOING FORWARD FOR THE ENSUING CALENDAR
10 YEAR. WHEN THAT WAS PART OF THE ADOPTED PROCESS BY
11 THE BOARD, WE THEN PROCEEDED WITH A ROLL-OUT OVER
12 TIME OF A NUMBER OF THE CONCEPT PLANS NEW AND
13 AMENDED THROUGHOUT CALENDAR 2025 WITH AN EYE TOWARDS
14 SEEING HOW THE PREFERENCE APPROACH WORKED WITH
15 RESPECT TO THE VARIOUS PROGRAMS AND WOULD COME BACK
16 IN A YEAR TO DISCUSS WITH THE BOARD THE RESULTS OF
17 THAT AND RECOMMENDATIONS GOING FORWARD FOR THE
18 FOLLOWING YEAR.
19 WE IN THAT TIME FRAME GOT THROUGH TWO
20 CLIN2 ROUNDS, TWO PDEV ROUNDS, AND ONE DISC4 ROUND.
21 AND THAT GAVE US A SET OF DATA ON HOW THE PREFERENCE
22 APPROACH WAS WORKING. WHAT WE DISCOVERED WAS THAT
23 THAT APPROACH CARRIED WITH IT A NUMBER OF UNINTENDED
24 CONSEQUENCES WHICH WERE CONVEYED BOTH BY THE BOARD
25 AND BY PI'S AND OTHER STAKEHOLDERS IN THE CIRM

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ECOSYSTEM TO THE TEAM. AND WE WERE INSTRUCTED TO INTEGRATE THOSE CONCERNS INTO OUR PROPOSAL FOR WHAT WE SHOULD DO THIS COMING YEAR.

AND SO THAT HAS RESULTED TODAY IN COMING BACK TO YOU WITH AN APPROACH WHICH DOES NOT USE THE PREFERENCES ANY FURTHER AND DOES INSTEAD USE A NEW APPROACH, INTEGRATING SCIENTIFIC REVIEW AT THE PRESELECTION PROCESS, WHICH ALLOWS US TO APPLY THE GUIDING PRINCIPLES AT THAT STAGE AS WELL AS AT THE BOARD ONCE FULL GWG REVIEW HAS BEEN DONE AND WILL HELP GUIDE THE BOARD IN ITS PROGRAMMATIC DISCUSSION ON WHICH GRANTS TO FUND AT ANY PARTICULAR GIVEN ROUND.

YOU WILL HEAR ON THE PROGRAM SIDE FROM SHYAM MUCH MORE ON THIS. GIL WILL GO OVER IN GREAT DETAIL HOW THE VOLUME CONTROL APPROACH TO REDUCING THE NUMBER OF THE APPLICATIONS TO A MORE MANAGEABLE AMOUNT WHILE STILL INCREASING THOSE THAT GO TO THE GWG AND ULTIMATELY THE BOARD FOR FULL CONSIDERATION IN ALIGNMENT WITH THE GUIDING PRINCIPLES. THEN SHYAM WILL COME BACK TO YOU WITH A DISCUSSION ON HOW THIS WILL ALL PLAY OUT AT THE ARS USING THIS NEW APPROACH.

WANTED TO SAY THAT THE PURPOSE OF THE NEW APPROACH IS TO MAINTAIN FOCUS ON OUR GOALS, TO BE

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1 FULLY TRANSPARENT WITH AN EYE TOWARDS INCREASED
2 FAIRNESS FOR ALL APPLICANTS, AND TO CONTROL THE
3 VOLUME OF THE GRANTS THAT ARE GOING THROUGH THE
4 SYSTEM IN A WAY THAT GIVES THE BOARD THE OPPORTUNITY
5 TO SELECT THOSE GRANTS THAT WILL GIVE PATIENTS THE
6 BEST CHANCE FOR THERAPEUTIC TREATMENTS GOING
7 FORWARD.
8 SO THAT'S AN INTRODUCTION HERE. I WANT TO
9 CLOSE BY GIVING MASSIVE THANKS TO ROSA, SHYAM, GIL,
10 AND THE TEAMS FOR ALL OF THE EXTREME AMOUNT OF HEAVY
11 LIFTING THEY'VE GONE TO GET US TO THIS POINT WHERE
12 WE CAN HAVE THE DISCUSSION TODAY, THE PREPARATION OF
13 THE MEMO, THE DECK, THE AMENDED CONCEPT PLANS,
14 EVERYTHING THAT WAS AGENDIZED FOR YOUR REVIEW IN
15 ADVANCE. SO THANK YOU ALL TO THE TEAM FOR THE
16 EXTREMELY DEDICATED EFFORT TO MAKE THIS HAPPEN. SO
17 WITH THAT, I'LL HAND IT OVER TO SHYAM.
18 DR. PATEL: THANK YOU, J.T., FOR THE
19 INTRODUCTION AND FOR THE OBLIGATORY BASEBALL
20 REFERENCE. I'M GOING TO SHARE THE SLIDE DECK, AND
21 THEN WE'LL GET GOING WITH THE PRESENTATION. GIVE ME
22 ONE SECOND PLEASE.
23 SO THANK YOU TO THE SCIENCE SUBCOMMITTEE
24 FOR THE OPPORTUNITY TO PRESENT TO YOU TODAY. AS
25 J.T. MENTIONED, WE'LL BE GOING OVER SEVERAL THINGS

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1 OVER THE COURSE OF THE NEXT TWO HOURS. I DO WANT TO
2 FIRST OFF ACKNOWLEDGING ROSA'S LEADERSHIP THROUGHOUT
3 THIS WHOLE PROCESS AS WELL AS THE PARTNERSHIP FROM
4 THE EXECUTIVE TEAM, GIL AND THE REVIEW TEAM, AS WELL
5 AS THE PROGRAMS COLLEAGUES, ESPECIALLY DR. JOE GOLD,
6 DR. KELLY SHEPARD, DR. SARA TAYLOR, AND DR. LIZ
7 NOBLIN, ALL OF WHOM ARE ON THE ZOOM WITH YOU TODAY.
8 SO AS WE GET GOING THROUGH THIS, AS J.T.
9 MENTIONED, I'LL START OFF SPEAKING ABOUT THE
10 PREFERENCES AND GUIDING PRINCIPLES. GIL IS GOING TO
11 GO THROUGH THE REVIEW PROCESS DETAILS, AND THEN
12 WE'LL COME BACK AROUND TO PROGRAMMATIC
13 RECOMMENDATION CONSIDERATIONS.
14 SO AS J.T. NOTED, OVER THE PAST FEW
15 MONTHS, THE BOARD HAS PROVIDED EXTENSIVE FEEDBACK,
16 MEASURED AS WELL AS CRITICAL, ON ASPECTS OF THE
17 PREFERENCES AND THE PREFERENCE-BASED EVALUATION
18 PROCESSES. WHILE THE BOARD DEFINITELY RECOGNIZED
19 THAT THERE IS A NEED TO HAVE FILTERING AND THAT CIRM
20 CANNOT FUND ALL PROJECTS, IT RAISED EXTREMELY VALID
21 CONCERNS THAT THE PREFERENCES WERE BEING USED IN A
22 WAY THAT COULD POTENTIALLY EXCLUDE CLASSES OF
23 MODALITIES OR INNOVATIVE PROJECTS AND THAT
24 APPLICATIONS WERE ULTIMATELY BEING TRIAGED WITHOUT
25 ANY SCIENTIFIC EVALUATION.

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1 THE BOARD ALSO PROVIDED EXTENSIVE FEEDBACK
2 ON GUIDING PRINCIPLES THAT MAY UNDERLIE EACH PROGRAM
3 AND TO MAKE CERTAIN THAT THOSE ARE APPROPRIATE FOR
4 THOSE PROGRAMS AND THAT THEY'RE TAILORED TO THOSE
5 PROGRAMS. AND ALSO IN ADDITION TO THAT, THE BOARD
6 STRESSED THE NEED TO HAVE TRANSPARENCY AS WELL AS
7 STRONGER COMMUNICATION ACROSS ANY OF THE PRIORITY
8 AREAS AND PROCESSES THAT ARE INCORPORATED INTO EACH
9 OF OUR FUNDING PROGRAMS.
10 AND LASTLY, THE BOARD HAD SEVERAL REQUESTS
11 FOR DATA, PARTICULARLY AROUND THE OUTCOMES OF THE
12 PREFERENCE PROCESS AS WELL AS THE CURRENT ACTIVE
13 AWARDS PORTFOLIO. ALL OF THAT IS IN THE MEMO AND IS
14 NOT NECESSARILY IN THIS PRESENTATION. WE'RE MORE
15 THAN HAPPY TO WALK THROUGH THAT.
16 SO OVER THE COURSE OF THIS PRESENTATION,
17 WE'LL BE PAUSING SEVERAL TIMES TO ENGAGE BOARD
18 FEEDBACK, TO GET YOUR FEEDBACK AND APPROVAL AND
19 AGREEMENT ON SEVERAL ELEMENTS AS WE GO THROUGH THIS.
20 FIRST OF ALL, A RECAP OF PREFERENCES. AS
21 J.T. MENTIONED, THE PREFERENCES REALLY FLOWED FROM
22 UNDERLYING GUIDING PRINCIPLES FOR EACH OF THE THREE
23 PROGRAMS THAT WE'RE GOING TO BE DISCUSSING TODAY,
24 DISC4, PDEV, AND CLIN2. AND THE INTENT WAS HOW ARE
25 THESE PROGRAMS GOING TO FUND PROJECTS THAT

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1 ULTIMATELY RESPOND TO THE APPROPRIATE IMPACT GOALS,
2 WHETHER THAT BE ADVANCING THERAPIES TO LATE STAGE
3 CLINICAL TRIALS OR RESULTING IN THE DISCOVERY OF
4 NOVEL BIOMARKERS AND TARGETS FOR DISEASES THAT
5 AFFECT HUMANS.
6 SO IN THAT SENSE, THE PREFERENCES WERE
7 INTENDED TO PLAY TWO KEY ROLES. AND THOSE WERE TO
8 HELP US PRIORITIZE WHAT WE FUND AND TO ALSO BE
9 APPLIED FOR VOLUME CONTROL SO THAT A MANAGEABLE
10 NUMBER OF APPLICATIONS WOULD GO TO GRANTS WORKING
11 GROUP REVIEW AND ULTIMATELY TO THE BOARD FOR
12 APPROVAL.
13 IN DOING BOTH OF THESE THINGS, THE CIRM
14 TEAM NEEDED TO MAKE SURE THAT THE PREFERENCES WERE
15 SPECIFIC, TRANSPARENT, AND COULD BE OBJECTIVELY
16 APPLIED. AS WE HAVE ALL LEARNED WITH RESPECT TO THE
17 BOARD FEEDBACK AS WELL AS THE ANALYSIS THAT'S IN THE
18 MEMO, THIS AMPLIFIED THE LIMITATIONS OF THIS
19 APPROACH. SO PARTICULARLY THAT THE PREFERENCES WERE
20 APPLIED PRIOR TO SCIENTIFIC REVIEW, AND SO THE
21 FILTERING FOR PRIORITY AREAS IS HAPPENING BEFORE THE
22 BENEFIT OF THE GRANTS WORKING GROUP SCIENTIFIC
23 REVIEW. AND SECONDLY, BY THEIR VERY NATURE, BEING
24 SO SPECIFIC AND DIRECTED, THE PREFERENCES WOULD
25 REQUIRE ANNUAL REVIEW OF OUR PORTFOLIO BY THE BOARD

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AS WELL AS ANNUAL ADJUSTMENTS.

ULTIMATELY WE CAME TO THE CONCLUSION, MUCH LIKE THE BOARD DID, THAT THIS IS NOT A SUSTAINABLE PROCESS IN THE LONG RUN FOR BUILDING A PORTFOLIO THAT IS THEN GOING TO BE COMPOSED OF IMPACTFUL PROJECTS THAT ULTIMATELY HELP US ACHIEVE OUR IMPACT GOALS.

AND SO IN EXTREMELY DRAMATIC FASHION, WE ARE PROPOSING TO REMOVE THE POINT-BASED PREFERENCES BOTH AS PREFERENCES THEMSELVES AS WELL AS THE POINT-BASED PREFERENCE VOLUME CONTROL MEASURE THAT WAS INTRODUCED IN THE DISC4, PDEV, AND CLIN2 PROGRAMS. INSTEAD, THE INTENT IS TO GO BACK TO THE GUIDING PRINCIPLES WHICH WERE PREVIEWED A LITTLE BIT IN JANUARY. WE'RE ACTUALLY GOING BACK TO THE PROGRAM GUIDING PRINCIPLES AND ENSURING THAT THE BOARD HAS AGREEMENT ON HOW THOSE GUIDING PRINCIPLES CAN SHAPE WHAT WE FUND FOR EACH OF THOSE THREE PROGRAMS AND ALSO HOW WE CAN APPLY THOSE GUIDING PRINCIPLES AT EACH STEP OF THE APPLICATION LIFE CYCLE.

SO THROUGHOUT THIS PRESENTATION, WE'RE GOING TO BE MAKING REFERENCE TO THE STEPS THAT ARE ON THIS SLIDE. I'M GOING TO TAKE JUST A MOMENT TO DESCRIBE THOSE SO THAT WE'RE ALL FAMILIAR WITH THE

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1 TERMINOLOGY THAT'S BEING USED HERE.
2 SO ONCE AN APPLICATION IS SUBMITTED, AS
3 J.T. MENTIONED, BECAUSE OF THE FACT THAT ALL OF OUR
4 PROGRAMS ARE AT VERY HIGH DEMAND, THERE'S A NEED TO
5 IMPLEMENT A VOLUME CONTROL STEP PRIOR TO THOSE
6 APPLICATIONS GOING TO FULL SCIENTIFIC REVIEW. THE
7 VOLUME CONTROL STEP FOCUSES AS WELL AS REDUCES THE
8 NUMBER OF APPLICATIONS TO ALLOW FOR MEANINGFUL
9 SCIENTIFIC REVIEW FOR EACH OF THOSE THREE PROGRAMS.
10 THE SECOND STEP IN THE PROCESS IS THAT
11 ONCE THOSE APPLICATIONS HAVE BEEN FORWARDED TO
12 SCIENTIFIC REVIEW, THE GRANTS WORKING GROUP, AS HAS
13 ALWAYS BEEN THE CASE, THAT THEY REVIEW AND THEY WILL
14 CONTINUE TO REVIEW THOSE APPLICATIONS GOING FORWARD,
15 WILL CONDUCT A SCIENTIFIC MERIT REVIEW OF THOSE
16 APPLICATIONS AND CREATE A SET OF FUNDING
17 RECOMMENDATIONS. THOSE FUNDING RECOMMENDATIONS AND
18 SCIENTIFIC REVIEW ARE FORWARDED TO THE ARS OF THE
19 ICOC WHICH INCORPORATES THE GWG RECOMMENDATION AS
20 WELL AS ANY NUMBER OF PROGRAMMATIC FACTORS TO MAKE
21 THEIR FUNDING DECISIONS.
22 SO THOSE ARE THREE STEPS THAT WE'LL BE
23 TALKING ABOUT THROUGHOUT THIS PROCESS. AND AS WE GO
24 FORWARD, WE'RE SEEKING TWO THINGS FROM THE BOARD
25 TODAY. WE'RE SEEKING YOUR BOARD FEEDBACK AS WELL AS

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1 AGREEMENT ON WHAT WE SHOULD FUND FOR EACH OF THESE
2 THREE PROGRAMS: DISC4, PDEV, AND CLIN2 EFFECTIVELY
3 WERE THE GUIDING PRINCIPLES UNDERLYING THESE
4 PROGRAMS.
5 AND SECONDLY, WE'RE SEEKING BOARD FEEDBACK
6 ON ENSURING THAT THE GUIDING PRINCIPLES ARE APPLIED
7 BY THE RIGHT PARTY WITH THE RIGHT INFORMATION AT THE
8 RIGHT TIME AT THOSE THREE CRITICAL STEPS OF THE
9 APPLICATION LIFE CYCLE.
10 ALONG THE WAY I'M GOING TO BE PAUSING
11 QUITE A BIT TO GET BOARD FEEDBACK ON EACH OF THE
12 GUIDING PRINCIPLES FOR EACH PROGRAM AS WELL AS ON
13 THE PROCESS.
14 SO I'M GOING TO JUMP RIGHT INTO THE
15 GUIDING PRINCIPLES FOR PDEV AND CLIN2. THESE ARE
16 THE PROGRAMS THAT ADVANCE PROJECTS FROM DISCOVERY
17 THROUGH ALL THE PRECLINICAL STUDIES TO GET TO IND
18 CLEARANCE AND THEN ALL STAGES OF CLINICAL TRIALS.
19 IN SETTING UP THE GUIDING PRINCIPLES FOR THIS, JUST
20 WANT TO TAKE A QUICK MOMENT TO GIVE A QUICK OVERVIEW
21 OF THE CURRENT LANDSCAPE OF CELL AND GENE THERAPIES
22 IN PARTICULAR. SO AS THE BOARD KNOWS, THERE ARE
23 ABOUT EQUAL NUMBERS OF APPROVED CAR-T THERAPIES, AAV
24 GENE THERAPIES, AND LENTIVIRAL MODIFIED HSC
25 THERAPIES. WHILE THE CAR-T THERAPIES ARE CURRENTLY

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1 FOCUSING ON BLOOD CANCERS, PARTICULARLY CD 19 AND
2 BCN-TARGETED THERAPIES. THE AAV AND LENTIVIRAL HSC
3 GENE THERAPIES HAVE A SLIGHTLY BROADER SCOPE,
4 TARGETING DIFFERENT DISEASE AREAS AND DIFFERENT
5 GENETIC BASES.
6 ACROSS THE BOARD, FOR THE MOST PART ALL OF
7 THE THERAPIES THAT HAVE BEEN APPROVED TO DATE HAVE
8 BEEN SHOWN TO HAVE SIGNIFICANT IMPACT ON CLINICAL
9 OUTCOMES FOR PATIENTS. AT THE SAME TIME ALL OF THEM
10 HAVE EXPERIENCED CHALLENGES IN PATIENT ACCESS. WHEN
11 WE LOOK AT THE PORTFOLIO OF PRECLINICAL AND CLINICAL
12 THERAPIES ACROSS THE SPECTRUM, NOT JUST CIRM, BUT
13 GLOBALLY, THERE ARE A LOT OF ENCOURAGING SIGNS.
14 THERE ARE QUITE A FEW DIFFERENT MODALITIES IN
15 GENETIC THERAPIES AND CELL THERAPIES BEING TARGETED
16 AT ALL MAJOR DISEASE AREAS.
17 IN THE CANCER SPACE YOU HAVE ALLOGENEIC,
18 AUTOLOGOUS, AND IN VIVO GENETIC THERAPIES BEING
19 TARGETED AT SOLID AND BLOOD CANCERS AND GENERATING
20 CLINICAL DATA.
21 ON THE IN VIVO SIDE, ALL THREE OF THE
22 CURRENT MOST ADVANCED GENE EDITING PLATFORMS,
23 CRISPR, BASE EDITING, AND PRIME EDITING ARE ALL
24 GENERATING CLINICAL DATA, AND SOME HAVE PRETTY
25 STRONG CLINICAL VALIDATION IN DIFFERENT DISEASE

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AREAS.

ANOTHER ENCOURAGING SIGN IS ON THE PLURIPOTENT STEM CELL-DERIVED THERAPIES WHERE AT THE MOMENT THERE ARE ADVANCED LATE STAGE CLINICAL TRIALS FOR THESE THERAPIES FOR PARKINSON'S DISEASE, TYPE 1 DIABETES, EPILEPSY, AND POTENTIALLY EVEN CARDIAC COMING SOON. THAT'S VERY ENCOURAGING. FIVE YEARS AGO I DON'T THINK WE WOULD HAVE THOUGHT THAT WAS EVEN POSSIBLE WHERE THE SPACE WAS AT THAT TIME.

AND SO PUTTING ALL OF THAT INTO PERSPECTIVE, THAT'S WHAT SHAPES THE GUIDING PRINCIPLES THAT YOU ARE GOING TO SEE ON THIS SLIDE. AND THEN I'LL PAUSE AFTER THAT TO GET BOARD FEEDBACK.

I'M GOING TO DISPLAY ALL OF THEM AT ONCE AND WALK THROUGH THEM. SO PLEASE BEAR WITH ME. SO THE GUIDING PRINCIPLES FOR THE PDEV PORTFOLIO AND THE CLIN2 PORTFOLIO ARE VERY SIMILAR IN SCOPE GIVEN WHAT I JUST MENTIONED ABOUT THE OVERALL LANDSCAPE OF CELL AND GENE THERAPIES. SO GIVEN THE FACT THAT THE BAR HAS BEEN SET PRETTY HIGH SO FAR, WE THINK THAT OVERALL THE PDEV PORTFOLIO AND THE CLIN2 PORTFOLIO SHOULD OFFER POTENTIAL FOR THERAPIES -- THE THERAPIES THAT WE FUND IN THESE PORTFOLIOS SHOULD OFFER POTENTIAL FOR TRANSFORMATIVE PATIENT OUTCOMES.

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1 AND ON THE PDEV SIDE, THEY SHOULD BE LEVERAGING
2 CUTTING-EDGE THERAPEUTIC TECHNOLOGIES TO SERVE THAT
3 PURPOSE OF TRANSFORMING PATIENT OUTCOMES.
4 ACROSS THE PORTFOLIO, PROJECTS THAT WE
5 FUND SHOULD LEARN FROM THE EXPERIENCES OF THE FIRST
6 GENERATION OF CELL AND GENE THERAPIES TO TAKE
7 MEANINGFUL, PRACTICAL, AND FEASIBLE STEPS TOWARD
8 ADDRESSING ACCESS AND AFFORDABILITY BARRIERS FOR
9 THOSE TYPES OF THERAPIES. IN ALIGNMENT WITH OUR
10 ACCESS AND AFFORDABILITY TOOLKIT AND REVIEW
11 PROCESSES AND SUPPORT MECHANISMS, THIS COULD RANGE
12 FROM ASPECTS OF THE THERAPEUTIC APPROACHES TO
13 PLANNING AT STAGE APPROPRIATE POINTS ALONG THE
14 DEVELOPMENT LIFE CYCLE FOR ACCESS AND AFFORDABILITY
15 OF THOSE THERAPIES, ALL OF WHICH, IN TURN, WOULD
16 HELP MAKE SURE THAT THOSE THERAPIES, IF THEY WERE TO
17 BE APPROVED, ARE IN BETTER POSITION TO BE ACCESSIBLE
18 FOR THE PATIENT POPULATION.
19 AND LASTLY, GIVEN THE FACT THAT AT THE
20 MOMENT CELL AND GENE THERAPIES ARE BEING TARGETED IN
21 ALL MANNER OF DIFFERENT DISEASES, OUR PORTFOLIO AS A
22 WHOLE FOR BOTH PDEV AND CLIN2 SHOULD BE BROADLY
23 ADDRESSING BOTH PREVALENT AND RARE DISEASES THAT
24 AFFECT CALIFORNIANS.
25 SO I'LL TAKE A BREAK THERE TO REQUEST

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1 BOARD FEEDBACK ON THE GUIDING PRINCIPLES FOR BOTH
2 THE PDEV PROGRAM AND THE CLIN2 PROGRAM. I'LL TURN
3 IT BACK OVER TO YOU, MARK.
4 CHAIRMAN FISCHER-COLBRIE: OKAY. WITH
5 THAT, IF YOU GUYS CAN HELP ME IF YOU SEE HANDS
6 RAISED BECAUSE I MAY NOT BE ABLE TO SEE THEM ALL
7 FROM MY SCREEN.
8 FIRST OF ALL, LET'S START WITH ANY
9 QUESTIONS OR COMMENTS WITH RESPECT TO WHAT SHYAM HAS
10 PRESENTED.
11 MR. TOCHER: CAROLYN MELTZER HAS HER HAND
12 RAISED. AND THEN PAT.
13 CHAIRMAN FISCHER-COLBRIE: CAROLYN.
14 DR. MELTZER: YEAH. SO THANK YOU, SHYAM.
15 I LIKE THROWING OUT THE POINT-BASED SYSTEM. I
16 THINK, WHILE WE ALL INTENDED IT TO BE HELPFUL, I
17 THINK IT'S NOT HAD -- IT'S HAD UNINTENDED
18 CONSEQUENCES AS YOU'VE POINTED OUT. AND I LIKE THE
19 IDEA OF GOING THROUGH THE PORTFOLIOS NOW TO FIGURE
20 HOW WE ENRICH THEM WITH WHICH TYPES OF GRANTS. SO
21 ANYWAY, JUST SOME SORT OF INITIAL THOUGHTS. I THINK
22 THIS IS GREAT.
23 CHAIRMAN FISCHER-COLBRIE: GREAT. PAT.
24 DR. LEVITT: YEAH. SO I THINK I'VE SAID
25 THIS BEFORE. I'LL JUST SAY IT TO THE WHOLE SCIENCE

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1 SUBCOMMITTEE THAT I KNOW THAT THE ACCESS AND
2 AFFORDABILITY COMPONENT IS A COMPONENT OF GRANTS
3 GOING, AND IT WAS UNCLEAR -- IT'S UNCLEAR TO ME HOW
4 THE GWG IS USING PLANS TO ADDRESS THIS. AND I THINK
5 THAT I HAVE NO ISSUE WITH HAVING ACCESS BEING JUDGED
6 IN TERMS OF THE PROPOSED RESEARCH THAT'S GOING TO BE
7 DONE LIKE EVENTUALLY ACCESS.
8 I STILL STRUGGLE WITH RESEARCH GROUPS
9 ADDRESSING AFFORDABILITY SINCE THAT IS A CHALLENGING
10 AREA THAT STATES AND FEDERAL OFFICIALS ARE
11 STRUGGLING WITH. AND TO HAVE INVESTIGATORS WEIGH IN
12 ON THAT, I THINK TAKES TIME. AND I DON'T QUITE -- I
13 STILL DON'T QUITE UNDERSTAND HOW THEY CAN ADDRESS
14 THAT IN A MEANINGFUL WAY FOR THE GWG TO EVALUATE.
15 I'LL STOP THERE. BUT ACCESS I BELIEVE THEY SHOULD
16 AND CAN ADDRESS.
17 CHAIRMAN FISCHER-COLBRIE: THANK YOU, PAT.
18 KEITH. OR, SHYAM, DO YOU WANT TO RESPOND TO THAT
19 COMMENT?
20 DR. PATEL: CERTAINLY. I CAN DO THAT. SO
21 THANK YOU, PAT, FOR THAT GREAT QUESTION. AND YOU
22 RAISE AN ELEMENT OF PRACTICALITY AND FEASIBILITY
23 WITH RESPECT TO THAT TOPIC. AND SO I'M GOING TO
24 ADDRESS IT FOR BOTH OF THESE PROGRAMS.
25 AND SO IN THE APPLICATION WE HAVE ASKED

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1 THE APPLICANT TO DEFINE THEIR CURRENT UNDERSTANDING
2 FOR THEIR PARTICULAR APPROACH WHAT IS THE
3 REIMBURSEMENT AND TREATMENT LANDSCAPE FOR THOSE
4 TYPES OF APPROACHES AND WHAT THEY PLAN TO DO IN
5 ORDER TO UNDERSTAND THAT BETTER OVER THE COURSE OF
6 THE AWARD AND WHERE THEY THINK THEY COULD BE MAKING
7 MEANINGFUL IMPROVEMENTS IN THAT SPACE.
8 AND SO ON THE PDEV SIDE, WHEN THE GRANTS
9 WORKING GROUP REVIEWS THAT, THE ACCESS AND
10 AFFORDABILITY COMPONENT IS PART OF THE OVERALL VALUE
11 PROPOSITION QUESTION THAT ASKS THEM TO CONSIDER THE
12 OVERALL IMPACT OF THAT THERAPY ON THE PATIENT
13 POPULATION, THE UNMET NEED, AS WELL AS WHETHER THE
14 AWARDEE UNDERSTANDS AND IS PLANNING TO ADDRESS THESE
15 BARRIERS GOING FORWARD IN A MEANINGFUL WAY. BUT
16 IT'S ONE COMPONENT OF THE OVERALL VALUE PROPOSITION
17 REVIEW PROCESS.
18 ON THE CLIN2 SIDE, THE ACCESS AND
19 AFFORDABILITY PLAN ELEMENTS ARE BAKED INTO WHAT THE
20 ACCESS AND AFFORDABILITY REVIEWERS LOOK AT. AND SO
21 BOTH IN THE CLIN2 AND THE PDEV SIDE, THE PLANNING
22 PART IS NOT MEANT TO BE PUNITIVE. IT'S ACTUALLY
23 MEANT TO BE WHAT'S YOUR CURRENT STATE, HOW ARE YOU
24 GOING TO USE CIRM FUNDING TO BETTER YOUR
25 UNDERSTANDING OF HOW THESE THERAPIES ARE DELIVERED

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1 TO PATIENTS AND HOW THEY'RE PAID. AND THEN WHAT ARE
2 MEANINGFUL STAGE-APPROPRIATE STEPS YOU ARE GOING TO
3 BE TAKING OVER THE COURSE OF THE CLINICAL
4 DEVELOPMENT TO MAKE SURE THAT, WHEN THIS THERAPY IS
5 CLOSER TO APPROVAL, THAT THE MARKET ACCESS, PATIENT
6 ACCESS ELEMENTS ARE ALSO COMING ALONG AT THE SAME
7 RATE.
8 SO THAT'S SORT OF HOW WE VIEW THIS IN THE
9 SENSE THAT DOES THE APPLICANT HAVE A GOOD
10 UNDERSTANDING. IF THEY DON'T, HOW DO THEY PLAN TO
11 ADVANCE THAT UNDERSTANDING OVER THE COURSE OF THE
12 CIRM AWARD? AND WHAT ARE SOME STEPS THAT THEY
13 PRACTICALLY TAKE AT THE MOMENT THAT THEY'VE ALREADY
14 DEFINED THAT COULD MAKE A DIFFERENCE HERE?
15 SO OVERALL WE THINK THAT IN THIS
16 PARTICULAR ELEMENT FOR ACCESS AND AFFORDABILITY,
17 THESE CAN BE VALUE ADDS TO AN ALREADY IMPACTFUL
18 PROJECT THAT MAY BE ATTRACTIVE FOR THE BOARD GOING
19 FORWARD.
20 CHAIRMAN FISCHER-COLBRIE: WITH THAT, IF I
21 MAY DROP IN A COMMENT. I THINK IT IS IMPORTANT TO
22 LEARN THE TERMINOLOGY BECAUSE ONE INDICATES ADDRESS.
23 AND ADDRESS IS AN OKAY TERM, BUT IT SOUNDS LIKE FOR
24 CONSIDERATION THE TERM MAYBE "ASSESS" IN THE
25 APPLICATION IS MORE DEFINITIVE ABOUT WHAT THE

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1 EXPECTATIONS ARE FOR EVALUATING THE CURRENT
2 LANDSCAPE AND, OF COURSE, IF THE PROPOSED MODALITY
3 IS HAVING A DIRECT IMPACT IN THE APPLICATION.
4 IS THAT PERHAPS A DEFINITIONAL ITEM THAT
5 MIGHT HELP CLARIFY WHAT IS MEANT BY ADDRESSING THE
6 BARRIERS?
7 DR. PATEL: SO I THINK, MARK, IF I
8 UNDERSTAND YOUR QUESTION CORRECTLY, IT'S MORE ABOUT
9 MAKING SURE THAT THE REVIEWERS ARE TRAINED AND
10 GUIDED ON HOW TO ASSESS THAT PARTICULAR QUESTION IN
11 THEIR CRITERIA. IS THAT APPROPRIATE TO SAY?
12 CHAIRMAN FISCHER-COLBRIE: YEAH. FIRST OF
13 ALL, SO THE APPLICANTS UNDERSTAND WHAT IS MEANT BY
14 ADDRESSING THE KNOWN BARRIERS, WHICH IS BY AND LARGE
15 WHAT I FUNCTIONALLY HEARD WAS AN ASSESSMENT PROCESS.
16 AND THE SECOND, THAT THE REVIEWERS UNDERSTAND THE
17 CONTEXT OF THIS. AND ON OCCASION, AS WE'VE
18 DISCUSSED, SOMETIMES SOME OF THE REVIEWERS THINK
19 IT'S A CONDITION OF, WELL, YOU'RE NOT ADDRESSING THE
20 UNDERLYING COST OF THIS MODALITY. AND THAT'S NOT
21 NECESSARILY AT ALL WHAT IS MEANT BY THIS BULLET
22 POINT. SO, THEREFORE, THERE'S DISCUSSION AND
23 TRAINING AND EDUCATION FOR THE REVIEWERS.
24 DR. PATEL: THANK YOU FOR THAT FEEDBACK.
25 CHAIRMAN FISCHER-COLBRIE: WITH THAT, WHY

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1 DON'T WE GO TO KEITH, I THINK, WAS NEXT AND THEN
2 JUDY HAD HER HAND UP EARLIER. KEITH.
3 DR. YAMAMOTO: THANKS, MARK. MY COMMENT
4 IS VERY SIMILAR TO PAT'S AS USUAL. AND I TALKED
5 ABOUT THIS IN THE BOARD MEETING BEFORE, AND THAT IS
6 A CONCERN ABOUT -- WELL, LET ME BACK UP AND SAY
7 FIRST THAT THIS CRITERION RUNS A BIG RISK OF
8 DISADVANTAGING NEW -- INNOVATIVE TECHNOLOGIES. NEW
9 TECHNOLOGIES AT THEIR BEGINNING ARE NEVER AFFORDABLE
10 BECAUSE THEY HAVE TO BE SCALED. AND I USE THE
11 EXAMPLE, THE OBVIOUS EXAMPLE, OF THE HUMAN GENOME
12 SEQUENCING THAT COST $3 BILLION AND TOOK TEN YEARS.
13 AND SO THAT WOULDN'T HAVE PASSED MUSTER VERY WELL
14 LOOKING AT AFFORDABILITY OR ACCESS SINCE IT WAS ONLY
15 ONE PERSON'S SEQUENCE. BUT THAT'S REALLY TRUE, THAT
16 GATE IS REALLY TRUE OF ALL NOVEL TECHNOLOGIES, ALL
17 INNOVATIONS.
18 SO I THINK IT WOULD BE REALLY UNFORTUNATE
19 IF THIS PRINCIPLE HAD THE CONSEQUENCE, DEFINITELY
20 UNINTENDED CONSEQUENCE, OF ELIMINATING INNOVATIVE,
21 NEW TECHNOLOGIES THAT ARE BROUGHT FORWARD. AND
22 THERE'S REALLY KNOW WAY THAT THE INVESTIGATORS COULD
23 MAKE A REASONABLE ARGUMENT THAT THEIR NOVEL
24 TECHNOLOGY IS AFFORDABLE.
25 AND THEN TALKING A BIT ABOUT WHAT MARK

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1 SAID, ASKING, AND I GUESS PAT DID THIS AS WELL, THAT
2 ASKING THE INVESTIGATORS TO ANTICIPATE HOW THE
3 PAYERS WOULD RESPOND TO ANY NEW TECHNOLOGY IN TERMS
4 OF WHETHER THEY'RE WILLING TO REIMBURSE OR NOT I
5 THINK IS NOT REALLY APPROPRIATE.
6 AND SO THE TWO CONCERNS I HAVE ABOUT THIS
7 SECOND PRINCIPLE IS THE EFFECT IT HAS ON INNOVATIVE
8 APPROACHES BECAUSE THEY ARE BY NECESSITY NOT
9 AFFORDABLE AT THE OUTSET. AND THAT ASKING
10 INVESTIGATORS, RESEARCHERS TO TRY TO ANTICIPATE THE
11 WAY THAT PAYERS WOULD RESPOND TO A NEW APPROACH, I
12 THINK, IS NOT APPROPRIATE.
13 VICE CHAIR BONNEVILLE: MARK, CAN I
14 RESPOND TO THAT?
15 CHAIRMAN FISCHER-COLBRIE: SURE.
16 VICE CHAIR BONNEVILLE: SO I THINK
17 SOMETHING THAT SHYAM, I THINK, WAS TRYING TO
18 EXPLAIN. SO IT IS NOT A PUNITIVE -- IT IS NOT
19 PUNITIVE FOR THE APPLICANTS AROUND THESE CRITERIA OF
20 ACCESSIBILITY AND AFFORDABILITY. IT IS REALLY MEANT
21 TO GENERATE A CONVERSATION FOR THEM INTERNALLY AS
22 WELL AS THE LIFE CYCLE OF THE AWARD GOES THROUGH
23 AROUND ACTIVITIES THAT WILL BENEFIT THEM WHEN
24 THEY'RE GOING FOR BLA AROUND -- IT'S NOT ARE THE
25 INSURERS GOING TO COVER IT. IT IS HAVE YOU STARTED

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1 CONVERSATIONS WITH INSURERS TO UNDERSTAND DATA THAT
2 THEY MIGHT NEED IN ORDER TO ONE DAY POTENTIALLY
3 REIMBURSE THIS THERAPY.
4 SO IT IS DEFINITELY NOT EVERYTHING HAS TO
5 BE FIGURED OUT AND SET BY THE TIME THEY'RE THERE.
6 IT IS HAVE YOU STARTED THE APPROPRIATE ACTIVITIES
7 THAT WOULD LEAD THIS TO ONE DAY PERHAPS BEING
8 REIMBURSABLE AND ALSO ACCESSIBLE TO MORE PEOPLE.
9 SO I WANT TO DISPEL ANY NOTION THAT THIS
10 SOMEHOW WORKS AGAINST THEM. THE ONLY WAY THIS WORKS
11 AGAINST THEM IS IF THEY PROVIDE NO INFORMATION OR
12 LOOK LIKE THEY DON'T WANT TO PARTICIPATE IN
13 ACTIVITIES AROUND THIS.
14 SO I DO THINK IT'S IMPORTANT FOR US AS A
15 FUNDING ORGANIZATION TO ASK THAT THIS BE SOMETHING
16 THAT IS CONSIDERED THAT THEY WORK TOWARDS BECAUSE,
17 AGAIN, WE WANT OUR THERAPIES TO BE ACCESSIBLE AND
18 ONE DAY REIMBURSABLE TO THE PEOPLE OF CALIFORNIA.
19 SO I HOPE THAT SORT OF EXPLAINS A LITTLE MORE. IF
20 WE NEED MORE EXPLANATION TO THE APPLICANTS AROUND
21 WHAT WE MEAN, BY ALL MEANS LET'S DO IT. AND IF
22 THAT'S WHAT WE'RE STRUGGLING WITH, THEN I THINK
23 SHYAM AND TEAMS CAN WORK TOWARDS THAT.
24 CHAIRMAN FISCHER-COLBRIE: YEAH, MARIA, I
25 THINK THAT'S IMPORTANT BECAUSE I THINK THIS IS A

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1 CONDITION WHERE WE'RE ALWAYS TRYING TO GET TO WITH
2 RESPECT TO MAKING AN ASSESSMENT AS OPPOSED TO SAYING
3 THAT, OKAY, OUR TECHNOLOGY IS GOING TO BE LOW COST
4 AND EASILY ADOPTED BY INSURANCE. IT'S MORE CREATING
5 A CHECKLIST FOR PEOPLE TO THINK ABOUT AS THEY MOVE
6 FORWARD IN THEIR CLINICAL AND PDEV APPLICATIONS.
7 LET'S CONTINUE WITH OTHER COMMENTS.
8 DR. YAMAMOTO: IF I COULD JUST INTERRUPT
9 FOR A SECOND.
10 CHAIRMAN FISCHER-COLBRIE: GO AHEAD,
11 KEITH.
12 DR. YAMAMOTO: I'M A LITTLE BIT CONCERNED
13 ABOUT -- WHAT I HEARD THERE IS THAT THERE'S AN
14 EXPECTATION FOR THE APPLICANTS, THE INVESTIGATORS,
15 TO HAVE AT LEAST INITIATED NEGOTIATIONS WITH PAYERS.
16 IS THAT REALLY WHAT WE ARE EXPECTING OR WANTING AT
17 THIS STAGE?
18 VICE CHAIR BONNEVILLE: AT THE CLIN STAGE?
19 IT DEPENDS ON WHICH STAGE YOU'RE TALKING ABOUT,
20 KEITH. AND I CAN HAVE -- I MEAN SHYAM CAN
21 ANSWER --
22 DR. YAMAMOTO: IT'S IN BOTH PDEV AND CLIN.
23 VICE CHAIR BONNEVILLE: IT'S DIFFERENT.
24 THERE ARE ACTIVITIES AT DIFFERENT STAGES OF THE
25 CYCLE. SO FOR PDEV, THE EXPECTATION IS DIFFERENT

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1 THAN LATER STAGE CLINICAL PROGRAMS.
2 DR. PATEL: YEAH. I CAN HIGHLIGHT SOME OF
3 THAT IF YOU'D LIKE. SO ON THE PDEV SIDE, IN THE
4 APPLICATION THEY'LL OFTEN DESCRIBE HOW THEIR
5 TECHNOLOGY PLATFORM IS MEANT TO REDUCE THINGS LIKE
6 MANUFACTURING COSTS OR THE USE OF REAGENTS OR EVEN
7 SOME ASPECTS OF THE DELIVERY OF THOSE THERAPIES IN A
8 CLINICAL SETTING WHERE IT COULD REDUCE COST BASED ON
9 THE SETTING WHERE THOSE THERAPIES ARE DELIVERED, HOW
10 THEY'RE DELIVERED, AND WHETHER IT'S USING EXISTING
11 PRACTICE OR NOT.
12 SO WE'RE SEEING A LITTLE BIT MORE OF THAT
13 SORT OF RICHER DIALOGUE FROM THE APPLICANTS IN THE
14 PROPOSALS.
15 AND THEN WITH RESPECT TO THE ACCESS AND
16 AFFORDABILITY PLANNING THAT MARIA IS REFERRING TO,
17 WE'VE INSTITUTED PLANNING GOALS ACROSS BOTH PROGRAMS
18 AND ARE MAKING CIRM FUNDING AVAILABLE FOR THEM TO
19 USE THAT TO MAKE THOSE GOALS. SO ON THE PDEV SIDE,
20 THE PLANNING GOAL IS THEY DON'T HAVE TO HAVE
21 ANYTHING DONE WHEN THEY APPLY; BUT OVER THE COURSE
22 OF THE AWARD, THEY CAN USE CIRM FUNDING TO BETTER
23 UNDERSTAND WHAT'S THE CURRENT TREATMENT AND
24 REIMBURSEMENT LANDSCAPE FOR EXISTING APPROACHES IN
25 THAT DISEASE AREA AS WELL AS UNDERSTANDING WHAT THE

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1 PATHWAY WOULD BE TO GET A THERAPY APPROVED AND
2 COMMERCIALIZED.
3 AND SO THEY'RE SORT OF DOING THE REALLY
4 EARLY PRODUCT DEVELOPMENT PLANNING AND CAN USE CIRM
5 FUNDING FOR THAT. AND THAT HELPS THEM BE BETTER
6 PREPARED WHEN THEY GET TO THE CLINIC.
7 ON THE CLINICAL SIDE, AS MARIA MENTIONED,
8 THERE'S STAGE-APPROPRIATE PLANNING ACTIVITIES THAT
9 THEY WOULD BE DOING. SO FOR A PHASE 1 TRIAL,
10 THEY'RE BUILDING ON THE PDEV WORK WHERE THEY'RE
11 FURTHER REFINING THEIR UNDERSTANDING OF THE
12 TREATMENT LANDSCAPE, THE REIMBURSEMENT LANDSCAPE, AS
13 WELL AS BUILDING ON THAT COMMERCIALIZATION PATHWAY,
14 MAYBE THINKING ABOUT MANUFACTURING IMPROVEMENTS AND
15 SO ON.
16 BUT AS THEY GET CLOSER TO PIVOTAL TRIALS,
17 THAT'S WHERE, AS MARIA NOTED, THERE SHOULD BE MORE
18 INTERACTION WITH PAYERS ON UNDERSTANDING HOW PAYERS
19 THINK ABOUT THESE TYPES OF THERAPIES SO THAT AT THAT
20 POINT WHEN THEY ARE APPROACHING APPROVAL, THAT
21 THEY'RE BETTER PREPARED TO BE ABLE TO ROLL THESE
22 THERAPIES OUT QUICKLY TO PATIENTS.
23 DR. YAMAMOTO: THANKS.
24 DR. MIASKOWSKI: MARK, COULD I COMMENT ON
25 THIS AS WELL?

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1 CHAIRMAN FISCHER-COLBRIE: YEAH. CHRIS
2 AND THEN WE'LL GET TO MONICA.
3 DR. CARSON: I WAS GOING TO COMMENT ON THE
4 SAME THING AS WELL.
5 DR. MIASKOWSKI: GO AHEAD, MONICA.
6 DR. CARSON: SO REALLY GREAT DISCUSSION.
7 SORRY TO CUT YOU OFF, BUT MAYBE I'LL SET UP YOUR
8 COMMENTS EVEN BETTER.
9 WHEN I'M LISTENING TO THIS, THIS IS A VERY
10 IMPORTANT DISCUSSION, BUT THERE'S TWO SEPARATE
11 ISSUES I JUST WANT TO PUT ON THE TABLE. FIRST ISSUE
12 IS WE'RE TALKING ABOUT THREE VERY DIFFERENT,
13 DISTINCT CRITERIA: SCIENTIFIC INNOVATION
14 TECHNOLOGIES, CLINICAL HEALTH IMPACT, AND ACCESS AND
15 AFFORDABILITY. ONE IS A BUSINESS, VERY DIFFICULT ON
16 SOCIETY, GOVERNMENT, AND LOTS ACCESSIBILITY TO
17 CLINICAL HEALTH IMPACT AND SCIENTIFIC INNOVATION.
18 AGAIN, REALLY TWO DIFFERENT EXPERTISE, BUT OFTEN
19 COMBINED IN STUDY SECTIONS THAT CAN BE DONE.
20 WHEN YOU LOOK AT THESE, YOU REALLY HAVE A
21 SITUATION WHICH GOES WRONG IN MANY FUNDING
22 FOUNDATIONS, NIH, AND THIS. WHAT PROGRAM MEANS, WHO
23 CAN REVIEW. AND LISTENING TO ALL THIS EXPLANATION
24 THAT I'M HEARING, BECAUSE I READ ALL THE DOCUMENTS,
25 REALLY PREPARED FOR THE MEETING, AND WHAT I'M

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1 HEARING WASN'T STRICTLY SPEAKING IN THE DOCUMENTS.
2 SO AN APPLICANT, A REVIEWER, AND A NEW GROUP OF
3 PEOPLE WHO IS ON THE BOARD WILL ALL COME TO
4 DIFFERENT THINGS HERE. AND SO THIS CONVERSATION IS
5 REALLY IMPORTANT. I LOVE ALL THE CRITERIA THAT ARE
6 BEING STATED, BUT TRYING TO DO ALL OF THEM IN SORT
7 OF THE SAME STEP, ISN'T THAT PUTTING SOMETHING AT
8 THE SECOND POINT ON THE TABLE?
9 SO THE FIRST POINT IS WE HAVE THREE THINGS
10 THAT ARE BEING SORT OF CONFOUNDED AND YOU'RE ASKING
11 FOR SOME STUFF THAT'S A MOVING TARGET, THAT, YES,
12 YOU WANT TO ENCOURAGE ACTIVITIES, BUT THE PEOPLE WHO
13 ARE GOING TO BE GOOD AT THE SCIENTIFIC INNOVATION
14 AND EVEN UNDERSTANDING THE CLINICAL IMPACT ARE NOT
15 THE BUSINESS FOLKS NOR SOME OF THESE OTHER FOLKS
16 THAT ARE GOING TO DO THE ACCESS AND ALL THAT.
17 SO HOW THOSE ARE ACTUALLY INTERPRETED ON
18 THE REVIEW REALLY NEEDS TO BE ARTICULATED WITH
19 REALLY GOOD EXAMPLES IN EACH OF THE DIFFERENT KINDS
20 OF STAGES OF THESE FUNDING MECHANISMS. I THINK
21 THAT'S PROBLEMATIC JUST LISTENING TO THE DISCUSSION,
22 BUT ASPIRATIONALLY WHERE WE NEED TO GO.
23 AND THE SECOND ONE IS NOT REALLY FOR NOW.
24 BUT WHEN WE'RE THINKING ABOUT THIS AND YOU'RE
25 THINKING ABOUT FUNDING, ARE WE TRYING TO GET GOOD

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1 TECHNOLOGY CLINICAL HEALTH IMPACT? AND THEN ARE WE
2 TRYING TO DO SOMETHING LIKE WHAT I USED TO WATCH THE
3 MS SOCIETY DO? PAYER INDUSTRY, THE FAST-FORWARD
4 KINDS OF ACCOUNTS WHERE YOU PAIR PEOPLE, AFTER IT
5 GOES TO THE SCIENTIFIC THINGS, YOU PAIR THEM OR THEY
6 COME IN WITH PAIR OF CHAMPIONS ON INDUSTRY THAT
7 SAYS, HEY, WE'RE GOING TO HELP GUIDE AND ADVISE.
8 SO I'M JUST PUTTING THAT ON THE TABLE,
9 SOMETHING FOR LATER DISCUSSIONS, BUT I THINK IT
10 ILLUSTRATES THAT THIS IS SOMETHING THAT'S BEEN
11 CONSIDERED BY OTHER THERAPEUTICS-ORIENTED
12 FOUNDATIONS THAT HAVE HAD IMPACT AND HAVE GUIDED
13 THINGS. AND I JUST THINK THE WAY WE'RE TALKING
14 ABOUT IT IS VERY GOOD. IT'S ASPIRATIONAL. BUT
15 DEVIL IS IN THE DETAILS HERE. THANK YOU. I HOPE,
16 CHRIS, THAT HELPS YOU SAY WHATEVER YOU ARE GOING TO
17 SAY.
18 DR. MIASKOWSKI: YEAH. THANKS, MONICA.
19 SO MY COMMENTS ARE PRIMARILY RELATED TO MY SERVICE
20 ON THE GRANTS WORKING GROUP. AND I BELIEVE THAT THE
21 ACCESS AND AFFORDABILITY PRINCIPLE IS EXCEEDINGLY
22 IMPORTANT. AND I WANT TO STATE THAT WE MAY BE
23 MISINTERPRETING THE CRITERIA. I THINK THE APPLICANT
24 GETS REALLY SPECIFIC GUIDANCE IN THE PDEV PROGRAM
25 AND IN THE CLIN PROGRAM ABOUT WHAT TO INCLUDE IN THE

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1 APPLICATION.
2 AND I CAN SHARE FROM THE MANY GRANTS I'VE
3 REVIEWED OR OBSERVED BEING REVIEWED ON THE GWG,
4 THERE'S A RANGE, JUST LIKE IN ANY OTHER GRANT
5 APPLICATION, OF RESPONSES. AND WE ADJUDICATE THOSE.
6 RECENTLY IN THE CLIN PROGRAM, THERE HAVE BEEN
7 EXPERTS ON THE GWG WHO CAN PROVIDE MEANINGFUL AND
8 RIGOROUS COMMENTS ABOUT THE APPLICANT'S APPRAISAL OF
9 ACCESS AND AFFORDABILITY.
10 SO I REALLY WANT TO ENSURE THE MEMBERS OF
11 THE COMMITTEE THAT THIS IS RIGOROUS AND AT THE SAME
12 TIME VERY, VERY FAIR. I DON'T THINK IT'S PENALIZING
13 ANYBODY.
14 I GUESS THE OTHER COMMENT I WOULD MAKE IS,
15 AS I'M HEARING THE DISCUSSION, IN OUR REVIEW OF
16 THESE APPLICATIONS, AFFORDABILITY DOES NOT EQUATE
17 WITH CHEAP. ALL RIGHT. WE HAVE HAD APPLICATIONS
18 WHERE THE APPLICANT HAS SAID WE'VE EVALUATED THE
19 LANDSCAPE, AND THIS THERAPY IS GOING TO COST X
20 SOMETIMES MILLIONS OF THE DOLLARS TO TREAT A
21 PATIENT. AND THE ARGUMENT IS ARTICULATED VERY, VERY
22 CLEARLY ABOUT THAT. AND NOBODY IS SAYING, OH, IT'S
23 CHEAP.
24 THE ACCESS ISSUE, PARTICULARLY FOR THE
25 CLIN PROGRAM, REALLY DOES REQUIRE THOUGHT. AND IN

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1 ADJUDICATING, CAN THEY RECRUIT THE PATIENTS? ARE
2 THEY LOOKING AT A BROAD AREA? DO THEY HAVE THE
3 RIGHT PEOPLE AT THE TABLE TO BEGIN TO BRING
4 PATIENTS, FAMILY MEMBERS INTO THE CLINICAL TRIAL?
5 ANYBODY WHO'S DOING A CLIN APPLICATION SHOULD HAVE
6 THAT. AND SOMETIMES WE DON'T SEE IT. AND THAT'S
7 NOT OKAY. WE CAN'T JUDGE WHETHER THAT THERAPY IS
8 GOING TO BE EVALUATED IN A RANDOMIZED TRIAL OR A
9 DOSE RESPONSE STUDY IN A MEANINGFUL WAY.
10 SO I WOULD SUPPORT ALL OF THE PRINCIPLES
11 THAT ARE LISTED HERE. AND I DO THINK THE COMMITTEE
12 HAS TO HAVE SOME TRUST THAT THE GWG HAS BOTH THE
13 SCIENTIFIC RIGOR AS WELL AS INDIVIDUALS ON THE
14 COMMITTEE WHO CAN JUDGE THE ACCESS AND
15 AFFORDABILITY.
16 CHAIRMAN FISCHER-COLBRIE: THANK YOU,
17 CHRIS. CAROLYN.
18 DR. MELTZER: YES. JUST BUILDING ON WHAT
19 CHRIS SAID, AND I DON'T SEE -- I'M NOT ON THE REVIEW
20 COMMITTEE, BUT I DO THINK THERE HAS TO BE A THOUGHT
21 PROCESS AS HOW IT WOULD BE LARGE-SCALE MANUFACTURED,
22 HOW IT COULD BE ACCESSIBLE AND AFFORDABLE.
23 NOW, I THINK YOU MADE THIS POINT, CHRIS.
24 IF IT'S A RARE DISEASE AND IT'S NEVER GOING TO BE
25 REALLY AFFORDABLE. IT STILL MAY BE IMPORTANT IF

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1 IT'S FOR SOLID TUMORS AND IT WILL NEVER BE
2 AFFORDABLE, THAT'S A DIFFERENT STORY BECAUSE OF THE
3 PREVALENCE ISSUE. SO I THINK OF IT AS A BALANCE AS
4 WELL.
5 I JUST WANT TO KNOW THAT THE REVIEWER IS
6 PROJECTING, AND THEN FOR THE CLIN2 IT HAS BEEN MORE
7 WORK CERTAINLY DONE IN THAT SPACE.
8 CHAIRMAN FISCHER-COLBRIE: LET CHRIS
9 FOLLOW UP AND THEN PICK UP WITH PAT.
10 DR. MIASKOWSKI: CAROLYN, I REALLY
11 APPRECIATED YOUR COMMENTS. AND WITHOUT DIVULGING
12 TOO MANY DETAILS, ONE OF THE APPLICATIONS WE
13 REVIEWED IN THE LAST CYCLE, THE APPLICANTS HAD
14 ENGAGED WITH THE INSURANCE INDUSTRY. AND I THINK
15 THEY DID A REALLY INTERESTING ANALYSIS. IT WAS A
16 CLIN2. AND WHAT I WAS STRUCK BY IN THAT APPLICATION
17 IS THAT THEY WERE INITIALLY GOING TO TEST THE
18 THERAPY IN INDIVIDUALS WHO HAD A SPECIFIC DISEASE
19 ENTITY. I'M NOT REMEMBERING ALL THE DETAILS, BUT IT
20 WOULD HAVE BEEN CONSIDERED A RARE DISEASE. AND THEY
21 WERE PROJECTING THAT THIS THERAPY WOULD GO FROM THIS
22 RARE DISEASE IMPLICATION TO THE BROADER POPULATION.
23 WHAT WAS FASCINATING TO ME REVIEWING THIS
24 FOR THE FIRST TIME WAS THE INSURANCE COMPANY SAID
25 WE'RE OKAY WITH THIS PRODUCT BEING INITIATED AND

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1 TESTED IN A RARE DISEASE AND IT'S GOING TO COST X
2 AMOUNT OF MONEY, AND WE'LL BE HAPPY TO CONSIDER IT
3 FOR REIMBURSEMENT.
4 HOWEVER, WHEN WE GET TO THE BROADER
5 GENERAL POPULATION OF ALL PEOPLE FOR THIS THERAPY,
6 WE'RE GOING TO RESERVE JUDGMENT. AND FOR ME I
7 REALLY APPRECIATED THAT THAT WAS A CONVERSATION CIRM
8 STARTED AND HOPEFULLY WILL CARRY OVER TO OTHER
9 THERAPIES. AND WE HAVE TO, I THINK, APPRECIATE AND
10 FOSTER THAT KIND OF DIALOGUE. IT'S GOING TO BE
11 REALLY, REALLY IMPORTANT FOR THESE THERAPIES.
12 CHAIRMAN FISCHER-COLBRIE: PAT.
13 DR. LEVITT: YEAH. SO, CHRIS, WHEN YOU
14 MADE YOUR FIRST COMMENT, YOU DID SOMETHING REALLY
15 IMPORTANT AT LEAST FROM MY PERSPECTIVE IS THAT YOU
16 ACTUALLY DESCRIBED ACCESS IN THE WAY THAT I THINK
17 ABOUT ACCESS. AND I'VE ENDORSED THAT BEING AN
18 IMPORTANT CRITERION BECAUSE THAT THE INVESTIGATORS
19 CAN DIRECTLY ADDRESS BECAUSE IT MATTERS FOR ALL THE
20 REASONS THAT YOU SAID. I DON'T HAVE TO STATE THEM
21 AGAIN.
22 AND SO I THINK HERE THE -- WHEN YOU SAY
23 ADDRESS BARRIERS TO ACCESS AND AFFORDABILITY, IT
24 PUTS THESE TWO THINGS TOGETHER THAT ACTUALLY REQUIRE
25 DIFFERENT ACTIVITIES IN ORDER TO MEET THE MISSION

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1 AND VISION OF THE ORGANIZATION. SO JUST USE CLEAR
2 LANGUAGE. DESCRIBE WHAT NEEDS TO BE ADDRESSED IN
3 TERMS OF ACCESS AND DESCRIBE WHAT NEEDS TO BE
4 ADDRESSED IN TERMS OF AFFORDABILITY. I HAVE FULL
5 CONFIDENCE IN THE GWG. I WANT TO HAVE THE SAME FULL
6 CONFIDENCE THAT A POTENTIAL APPLICANT UNDERSTANDS
7 WHAT THE GWG IS LOOKING FOR. AND SO IT'S JUST A
8 MATTER OF SENTENCES THAT GET WRITTEN IN A DIFFERENT
9 WAY THAT MAKE IT CLEAR HERE'S WHAT WE MEAN BY YOUR
10 ENGAGEMENT IN TERMS OF ACCESS, AND HERE'S WHAT WE
11 MEAN BY YOUR ENGAGEMENT IN AFFORDABILITY. AND IT
12 CAN BE IN A BULLET.
13 I'M NOT ASKING FOR ALL OF THE EXPLANATION
14 TO BE IN THE BULLET. THESE ARE GUIDING PRINCIPLES.
15 BUT I DO THINK THAT THEY ARE SEPARATE, AND YOU
16 DESCRIBED THE SEPARATE ACTIVITIES THAT ARE GOING ON.
17 RIGHT NOW IT BASICALLY LOOKS LIKE WHAT ARE THE
18 BARRIERS FOR THESE THINGS. AND AS AN INVESTIGATOR,
19 LIKE SOME INVESTIGATORS MAY BE ABLE TO DO WHAT YOU
20 JUST DESCRIBED IN TERMS OF THAT EXAMPLE. OTHER
21 INVESTIGATORS THAT I KNOW, WHO I'VE TALKED TO, JUST
22 GIVE THEM THIS LANGUAGE. HOW DO YOU INTERPRET THIS?
23 WELL, I DON'T HAVE ACCESS TO THE LEGISLATURE TO TALK
24 ABOUT MEDICAID REIMBURSEMENT AND WHAT THE ISSUES ARE
25 THERE. BUT I CAN GO TO MY CEO WHO IS TALKING TO

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1 THEM ABOUT IT.
2 SO I DO THINK EMPHASIZING THE DIFFERENT
3 ACTIVITIES AND WHAT ACTUALLY YOU WANT, WHAT WE WANT
4 FROM THE APPLICANT IS REALLY CRITICAL. AND THEN I
5 THINK THERE WON'T BE ANY CONFUSION, AND EVERYONE
6 WILL BE HAPPY ABOUT WHAT'S GETTING ENDORSED. SO
7 SOME WORDSMITHING NEEDS TO OCCUR.
8 DR. MIASKOWSKI: BUT I THINK, PAT, FROM MY
9 EXPERIENCE WITH THIS, AND MAYBE OTHERS CAN COMMENT
10 AS WELL, I THINK THE QUESTIONS ON THE APPLICATION
11 ARE CLEAR. THEY'RE NOT AMBIGUOUS. SO MAYBE WE NEED
12 TO LOOK AT THEM IN MORE DETAIL, BUT THEY ARE
13 SEPARATE ON THE APPLICATION AND PEOPLE RESPOND.
14 DR. LEVITT: RIGHT. BUT THESE GUIDING
15 PRINCIPLES ARE GOING TO GO OUT IN THE CALLS FOR AND
16 THE REQUEST FOR APPLICATIONS. THESE ARE THE GUIDING
17 PRINCIPLES. THIS IS THE LANGUAGE. AND SO MAYBE
18 THEY DIG DEEPER AND THEY'LL GET CLARITY. I THINK
19 IT'S PRETTY STRAIGHTFORWARD TO DO SOME EDITING HERE
20 ON THIS ONE, AND THEN WE CAN MOVE FORWARD.
21 DR. PATEL: MAY I COMMENT?
22 CHAIRMAN FISCHER-COLBRIE: GO AHEAD,
23 SHYAM. YEAH, PLEASE. THANK YOU.
24 DR. SAMBRANO: SORRY. THIS IS GIL. SO
25 ONE OF THE THINGS I JUST WANT TO CLARIFY. THE

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1 GUIDING PRINCIPLES THAT WE'RE PRESENTING HERE ARE
2 THE HIGHEST LEVEL THAT APPLY TO CIRM. SO THIS IS
3 WHAT CIRM DOES, NOT WHAT WE EXPECT APPLICANTS TO DO.
4 THE GUIDING PRINCIPLES INFORM ULTIMATELY
5 THE REVIEW CRITERIA. THEY INFORM THE OBJECTIVES OF
6 THE PROGRAMS THAT WE HAVE. AND SO THE INSTRUCTIONS
7 WILL FOLLOW THOSE. AND SO WHEN IT COMES TO ACCESS
8 AND AFFORDABILITY, WE DO HAVE A PRETTY DETAILED
9 SPREADSHEET THAT OUTLINES VERY SPECIFICALLY ACCESS
10 ELEMENTS VERSUS AFFORDABILITY ELEMENTS THE
11 APPLICANTS AT DIFFERENT STAGES ARE EXPECTED TO MEET.
12 AND IN MANY CASES, AS SHYAM POINTED OUT, ESPECIALLY
13 FOR THE PDEV PROGRAM, THERE'S REALLY NO EXPECTATION
14 GOING IN. IT'S ONLY AS YOU GET TO THE LATER STAGES.
15 AND WHEN IT COMES TO THE GRANTS WORKING
16 GROUP MEMBERS, THE GRANTS WORKING GROUP MEMBERS ALL
17 GO THROUGH TRAINING SESSIONS. WE HAVE A LOT OF
18 TIMES ONE-ON-ONES WITH THEM TO TALK ABOUT THE
19 OVERALL PROGRAM, THE REVIEW CRITERIA, AND SO ON.
20 BUT WE HAVE REVIEWERS THAT ARE NOT NECESSARILY PART
21 OF THE GRANTS WORKING GROUP THAT ARE ACCESS AND
22 AFFORDABILITY EXPERTS THAT HELPED REVIEW
23 SPECIFICALLY THESE DOCUMENTS AND GIVE US CLUES ABOUT
24 WHAT IT IS THAT THE APPLICANTS CAN DO TO IMPROVE
25 THEIR PROGRAM AND KIND OF JUST TELL US WHAT STATUS

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1 THEY ARE AT WITH THE GOAL OF PROVIDING ENOUGH
2 INFORMATION THAT GOES BACK TO THE APPLICANT TO CIRM
3 SUCH THAT IF THERE'S AN AWARD, WE KNOW HOW TO HELP
4 THEM AND PROVIDE ADDITIONAL GUIDANCE.
5 SO JUST WANT TO MAKE SURE THAT IT'S
6 UNDERSTOOD THAT THERE'S DEFINITELY TRAINING, THERE'S
7 DEFINITELY A SET OF LANGUAGE AND CLEAR PARAMETERS
8 FOR HOW TO ADDRESS THIS.
9 DR. PATEL: AND THEN ONE ADDITIONAL POINT
10 TO THAT. ONCE THE AWARDS ARE APPROVED ON THE CLIN2
11 SIDE IN PARTICULAR WHERE THERE ARE THESE ESCALATING
12 SORT OF ACTIVITIES THAT NEED TO BE DONE, THE CLIN2
13 TEAM WILL WORK WITH EXTERNAL EXPERTS, BRING THEM IN
14 TO ADVISE THE AWARDEE ON SOME OF THE FEEDBACK THAT'S
15 BEEN PROVIDED AND WHAT PRACTICAL STEPS THEY CAN TAKE
16 OVER ON THE COURSE OF THE CLIN2 AWARD TO ADVANCE
17 THEIR PROGRAMS WITH RESPECT TO PLANNING FOR EVENTUAL
18 ACCESS.
19 DR. LEVITT: I GET ALL THAT, AND I
20 UNDERSTAND THE REVIEW PROCESS. I HAVEN'T SEEN THE
21 QUESTIONS. I'M LOOKING AT THESE TWO BOXES IN THE
22 CONTEXT OF WHAT YOU'RE PROPOSING TO DO IS TO USE
23 THESE CRITERIA TO MAKE DECISIONS ABOUT GRANTS MOVING
24 FORWARD OR NOT MOVING FORWARD. NOT ABOUT THE REVIEW
25 CRITERIA, NOT ABOUT WHAT HAPPENS AFTER A DECISION IS

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1 MADE ABOUT FUNDING OR NOT. I GET ALL THAT. AND
2 THERE'S GOING TO BE GREAT GUIDANCE, ET CETERA, ET
3 CETERA. BUT WHAT WE'RE ADDRESSING HERE TODAY IS THE
4 LANGUAGE HERE THAT IS GOING TO BE USED TO DETERMINE
5 WHETHER, AND YOU'LL GET TO IT, AS WHETHER AN
6 APPLICATION IS GOING TO MOVE FORWARD OR NOT. NOT
7 ABOUT ANYTHING ELSE, NOT ABOUT WHAT HAPPENS WHEN THE
8 REVIEW OCCURS, NOT ANYTHING.
9 SO THE CONTENT NEEDS TO -- OF WHAT'S GOING
10 TO BE REVIEWED NEEDS TO ADDRESS THESE THINGS. AND
11 I'M JUST ASKING FOR CLARITY AROUND THEM. NOT TO
12 TAKE IT OUT, BUT TO JUST MAKE IT CLEAR AND NOT WAIT
13 UNTIL, WELL, ONCE WE GET IT REVIEWED AND THEY'RE
14 GOING TO GET FUNDED, THEN WE'RE GOING TO HAVE MORE
15 ADVICE FOR THEM. THAT'S GREAT. I'M NOT CRITICIZING
16 THAT. I'M JUST TALKING ABOUT THE PROCESS, WHICH IS
17 WHAT WE'RE ADDRESSING HERE TODAY, OF HOW A DECISION
18 IS MADE BY THE GWG ABOUT SOMETHING MOVING FORWARD OR
19 NOT. AND THERE'S A HUGE SENSITIVITY BY
20 INVESTIGATORS, AND WE HAVE TO ADDRESS THAT IN A
21 REALLY CLEAR WAY, REALLY CLEAR. AND SO THESE WORDS
22 MATTER RIGHT NOW BECAUSE THAT'S WHAT THEY'RE GOING
23 TO BE SEEING. THEY'RE GOING TO BE SEEING THIS, AND
24 THEN THEY'LL GET TO THE APPLICATION. BUT THEY'RE
25 GOING TO WANT TO KNOW WHY OR HOW MY GRANT MOVES

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1 FORWARD OR NOT.
2 AND SPEAKING AS SOMEBODY WHO CONTINUES TO
3 APPLY FOR GRANTS, THIS IS REALLY IMPORTANT TO BE
4 CLEAR. AND WE DON'T WANT TO DEAL WITH, AGAIN, THE
5 ISSUES THAT HAVE BEEN RAISED PUBLICLY BY
6 INVESTIGATORS THAT POINTED OUT SOME OF THE PROBLEMS
7 IN THE INITIAL APPROACH TO CONTROLLING NUMBERS.
8 EVERYONE HERE AND ON THE BOARD AGREES AND I THINK
9 INVESTIGATORS UNDERSTAND THAT SOME MECHANISM HAS TO
10 OCCUR. THIS IS A GREAT STEP. I'M JUST ASKING FOR
11 SOME SIMPLE, CLEAR LANGUAGE ABOUT WHAT YOU WANT IN
12 BULLET NO. 2. THAT'S ALL.
13 DR. PATEL: THAT'S REALLY HELPFUL, PAT.
14 SO HERE, JUST TO -- I THINK THERE'S GOING TO BE
15 LAYERS AS WE GO FORWARD. HERE WE'RE JUST SAYING IF
16 WE'RE GOING TO CONSTRUCT A PORTFOLIO FOR PDEV AND
17 CLIN2, ON THE WHOLE THE PROJECTS SHOULD BE
18 ADDRESSING THESE THINGS. RIGHT? AND SO IF WE'RE
19 LOOKING AT THE MAKEUP OF THE ENTIRE PORTFOLIO, I
20 THINK AS WE GET LATER ON, WE'RE GOING TO BE TALKING
21 ABOUT THE SELECTION CRITERIA AND THE REVIEW CRITERIA
22 AS WELL. AND I THINK AT THAT POINT YOUR INPUT IS
23 REALLY VALUABLE AS TO HOW WE CAN TIGHTEN UP THOSE
24 CRITERIA TO MAKE SURE THAT IT'S CLEAR TO THE
25 APPLICANT AND TO THE REVIEWER BECAUSE -- AND ALL OF

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1 THAT IS GOING TO BE IN THE PA AS WELL AS THE GUIDING
2 PRINCIPLES, THE SELECTION CRITERIA, THE REVIEW
3 CRITERIA, AS WELL AS THE SELECTION PROCESS IN TERMS
4 OF HOW EVERYTHING IS SCORED.
5 BUT I AGREE WITH YOU. IT'S IMPORTANT TO
6 MAKE SURE THAT EVERYBODY HAS A COMMON UNDERSTANDING
7 OF WHAT WE MEAN WHEN WE SAY THESE TYPES OF TERMS.
8 CHAIRMAN FISCHER-COLBRIE: CHRIS.
9 DR. MIASKOWSKI: COULD I ASK A DIFFERENT
10 QUESTION --
11 CHAIRMAN FISCHER-COLBRIE: OF COURSE.
12 DR. MIASKOWSKI: -- IF WE'RE DONE WITH THE
13 ACCESS AND AFFORDABILITY? I HAVE LESS PROBLEM
14 WITH --
15 CHAIRMAN FISCHER-COLBRIE: JUST REAL
16 QUICK, CHRIS. I CONCUR WITH PAT'S STATEMENT, THAT
17 WE NEED TO DO ADDITIONAL WORK WITH THOSE PARTICULAR
18 BULLET POINTS BECAUSE THE CLARITY AT THE TOP LEVEL
19 WILL DRIVE A LOT OF CONCERN OR ANXIETY IF THERE'S
20 TOO MANY INTERPRETATIONS THAT CAN BE APPLIED TO
21 THAT.
22 AND ALSO, I WANT TO COMPLIMENT THE CIRM
23 TEAM BECAUSE I THINK THE UNDERLYING STEPS WITH THE
24 MATRIX OF DECISION POINTS AND HOW IT'S ALL LAID OUT,
25 THE ADDITIONAL EXPERTS ON GWG IS A VERY COMPETENT

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1 REVIEW CYCLE. SO JUST WANTED TO ADD THOSE COMMENTS.
2 CHRIS, GO AHEAD.
3 DR. MIASKOWSKI: SO AS YOU KNOW, I HAVE
4 LESS DISAGREEMENT WITH ACCESS AND AFFORDABILITY.
5 THE FIRST CRITERIA, I'M STRUGGLING WITH, AS SOMEONE
6 WHO REVIEWS THESE GRANTS AND TRYING TO THINK ABOUT
7 THIS IF I WERE AN APPLICANT SUBMITTING THEM, HOW
8 WE'RE THINKING ABOUT WHAT A TRANSFORMATIVE
9 IMPROVEMENT IS. I'D LOVE TO HEAR SOME COMMENT ON
10 THAT. I WOULD ACKNOWLEDGE THAT IT PROBABLY DEPENDS
11 ON THE THERAPY THAT'S BEING INITIATED, BUT COULD YOU
12 COMMENT ON THAT, SHYAM, OR OTHERS ON THE TEAM?
13 DR. PATEL: YES. SO FOR BOTH PROGRAMS,
14 THE TRANSFORMATIVE IMPROVEMENT COMES INTO THE VALUE
15 PROPOSITION REVIEW CRITERIA. AND GIL CAN GET INTO
16 THAT A LITTLE BIT MORE. BUT BOTH IN THE APPLICATION
17 REVIEW CRITERIA, WHAT WE'RE ASKING THE APPLICANT TO
18 DO IS DESCRIBE WHAT IS THE CURRENT STATE OF
19 TREATMENTS, BOTH THAT ARE APPROVED AND IN
20 DEVELOPMENT, FOR THAT PARTICULAR INDICATION. AND
21 THEN HOW IS YOUR THERAPY IMPROVING UPON THOSE IN
22 TERMS OF CLINICAL OUTCOMES FOR THOSE PATIENTS?
23 AND EVEN IN THE -- THERE'S QUESTIONS ABOUT
24 UNMET MEDICAL NEED, AND THEY'RE ALSO FRAMED IN THE
25 WAY OF THERE'S AN UNMET MEDICAL NEED. HOW IS YOUR

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1 THERAPY ADDRESSING THIS UNMET MEDICAL NEED IN A
2 MEANINGFUL WAY BEYOND WHAT ELSE IS CURRENTLY
3 AVAILABLE TO THOSE PATIENTS?
4 AND SO IN THAT SENSE, IT'S GOING TO BE
5 SPECIFIC TO THAT INDICATION, BUT IT'S MEANT TO BE
6 SOMETHING THAT'S NOT JUST AN INCREMENTAL
7 IMPROVEMENT, BUT SOMETHING THAT IS REALLY GOING TO
8 MAKE A DIFFERENCE IN THAT PATIENT'S LIFE. AND THEY
9 HAVE TO BE ABLE TO EXPLAIN THAT WITH RESPECT TO WHAT
10 ELSE IS AVAILABLE TO THAT PATIENT NOW OR MAY BE
11 AVAILABLE TO THAT PATIENT IN A CLINICAL TRIAL.
12 DR. MIASKOWSKI: THANK YOU.
13 CHAIRMAN FISCHER-COLBRIE: OTHER
14 QUESTIONS, COMMENTS, OR FEEDBACK TO THE TEAM? I
15 DON'T SEE ANY HANDS RAISED. SO WITH THAT, WE'RE
16 GOING TO PROCEED TO THE NEXT AGENDA ITEM.
17 DR. PATEL: THANK YOU FOR YOUR THOROUGH
18 AND VERY WELL THOUGHTFUL FEEDBACK ON BOTH OF THESE
19 PROGRAMS. WE'LL TAKE INTO CONSIDERATION AND COME
20 BACK TO YOU ON THAT SECOND BULLET POINT. I THINK WE
21 CAN ALL AGREE THAT THE PATIENT ACCESS COMPONENT IS
22 UNIVERSAL ACROSS BOTH PROGRAMS, BUT POTENTIALLY
23 CLARIFYING THAT POINT MAY BE THE APPROPRIATE WAY TO
24 GO.
25 CHAIRMAN FISCHER-COLBRIE: THANK YOU,

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1 SHYAM.
2 DR. PATEL: SO WE'RE GOING TO MOVE ON TO
3 THE DISC4 PROGRAM GUIDING PRINCIPLES, AND I'M GOING
4 TO TAKE A MINUTE AND DESCRIBE THIS PROGRAM. SO AS
5 THE BOARD MAY RECALL, THE DISC4 PROGRAM EVOLVED FROM
6 THE RE-MIND PROGRAM. AND THE INTENT HERE IS TO
7 FOSTER MULTIDISCIPLINARY AND INNOVATIVE APPROACHES
8 TO BETTER UNDERSTANDING OF THE MECHANISMS BEHIND
9 HUMAN DISEASES ULTIMATELY RESULTING IN NOVEL
10 BIOMARKERS OR TARGETS THAT COULD THEN DOWNSTREAM
11 LEAD TO BETTER THERAPIES.
12 SO THE REAL FOCUS IS ON MULTIDISCIPLINARY
13 TEAMS COMING TOGETHER AND ADDRESSING -- LEVERAGING
14 ALL THE DATA THAT THEY HAVE AVAILABLE AND CAN
15 GENERATE TO BETTER UNDERSTAND DIFFERENT DISEASE
16 MECHANISMS AND THEN TO TAKE THAT TO THE NEXT STEP TO
17 DEVELOP NOVEL BIOMARKERS AND NOVEL TARGETS FOR THOSE
18 DISEASES.
19 AND SO IN THAT SENSE, THE DISC4 GUIDING
20 PRINCIPLES ARE MEANT TO FOSTER THAT. AND SO DISC4
21 PORTFOLIO AS A WHOLE WILL CREATE MULTIDISCIPLINARY
22 RESEARCH APPROACHES THAT INTEGRATE DIVERSE SOURCES
23 OF EVIDENCE. THEY'LL INNOVATE THROUGH
24 COLLABORATION, NETWORK SYNERGY, AND DATA LEVERAGE.
25 AND WHAT WE MEAN BY THAT IS THAT NOT ONLY IS EACH

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1 AWARD MULTIDISCIPLINARY IN NATURE, BUT THE DISC4
2 AWARDEES CAN LEVERAGE COMMONALITIES BETWEEN
3 DIFFERENT PROJECTS AND BE ABLE TO COMBINE THEIR
4 KNOWLEDGE, COMBINE THEIR DATA, AND THEIR RESOURCES
5 TO ADVANCE THE FIELD AS A WHOLE.
6 AND LASTLY, THAT THE DISC4 PORTFOLIO WILL
7 IMPLEMENT NEURO TASK FORCE AND OTHER ORGANIZATIONAL
8 PRIORITIES. SO AS YOU MAY RECALL, THE DISC4 PROGRAM
9 HAS A CLEAR FOCUS AREA. AND SO THIS INTENT, THE
10 PORTFOLIO SHOULD BE RESPONSIVE TO THOSE FOCUS AREAS
11 AS THEY EVOLVE ON AN ANNUAL BASIS.
12 AND I'LL PAUSE THERE FOR BOARD FEEDBACK ON
13 THE DISC4 GUIDING PRINCIPLES.
14 CHAIRMAN FISCHER-COLBRIE: OKAY. IF YOU
15 CAN SEE HANDS FOR ME, THAT WOULD BE GREAT.
16 MR. TOCHER: MARIA'S HAND IS RAISED.
17 VICE CHAIR BONNEVILLE: SHYAM, SO YOU'RE
18 SUGGESTING THAT THIS PROGRAM, WHICH I BELIEVE THIS
19 IS WHAT WE DID LAST TIME, WOULD BE FOCUSED ON ONE
20 GENERAL AREA. AND THEN ARE YOU ASKING THE BOARD OR
21 THE SCIENCE SUBCOMMITTEE AND THEN THE BOARD TO EVERY
22 YEAR WHEN THIS PROGRAM WOULD COME UP, THE FOCUS AREA
23 WOULD COME TO THE SCIENCE SUBCOMMITTEE AND THEN THE
24 BOARD, AND THAT'S WHAT WE WOULD AGREE, AND WE WOULD
25 NARROWLY FOCUS THIS. SO THIS WOULD NOT BE OPEN

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1 GENERALLY. THIS WOULD BE THE PROGRAM WHERE WE
2 FOCUSED IN SPECIFICALLY ON ONE MODALITY OR WHATEVER
3 IT MIGHT BE. IS THAT CORRECT?
4 DR. PATEL: I'M GOING TO TAKE THAT
5 QUESTION IN MULTIPLE POINTS. SO FIRST OF ALL --
6 VICE CHAIR BONNEVILLE: MULTIPLE?
7 DR. PATEL: THAT WAS A GREAT QUESTION, AND
8 I SHOULD HAVE PROVIDED A LITTLE BIT MORE CONTEXT
9 THERE. SO TOWARD THE END OF THIS PRESENTATION, WE
10 WILL BE ASKING THE BOARD TO PROVIDE FEEDBACK AND
11 AGREEMENT ON THE NEXT FOCUS AREA. SO RIGHT NOW THE
12 WAY THIS PROGRAM IS IMPLEMENTED IS THAT THERE IS A
13 FOCUS AREA, BUT IT'S NOT EXCLUSIVE.
14 VICE CHAIR BONNEVILLE: OKAY.
15 DR. PATEL: SO IN THAT SENSE, THE FIRST
16 ROUND OF THE DISC4 PROGRAM HAD A FOCUS AREA OF
17 NEUROLOGICAL DISORDERS. AND SO THAT WAS THE FOCUS
18 AREA, BUT IT WAS NOT EXCLUSIVE TO ACCEPTING
19 APPLICATIONS THAT ONLY ADDRESS THAT PARTICULAR
20 DISEASE AREA. OTHERS COULD HAVE SUBMITTED.
21 BY AND LARGE, MOST OF THE APPLICATIONS
22 THAT CAME IN WERE NEURO, AND ALL THE ONES
23 THAT -- THERE'S GOING TO BE -- I'M NOT GOING TO SAY
24 ANYTHING MORE BECAUSE THAT PROGRAM IS UNDER CURRENT
25 REVIEW. BUT MOST OF WHAT WE GOT WAS IN THE NEURO

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AREA. I THINK IT'S IN THE MEMO. TODAY WHAT WE'RE REQUESTING FROM THE BOARD LATER ON IN THIS PRESENTATION IS TO UPDATE THE FOCUS AREA FOR THE NEXT ROUND.

VICE CHAIR BONNEVILLE: OKAY.

DR. PATEL: HOWEVER, AT THIS POINT WE'RE NOT ASKING THE BOARD TO MAKE IT EXCLUSIVE. BUT IF THAT IS SOMETHING THE BOARD WOULD LIKE TO CONSIDER, WE'RE OPEN TO THAT DISCUSSION. AND DR. KELLY SHEPARD, WHO MANAGES THIS PROGRAM, IS ON THE LINE AS WELL.

VICE CHAIR BONNEVILLE: BUT IT IS THE TEAM'S FEELING THAT, IF IT WAS EXCLUSIVE, THE PROGRAM WORKS BETTER BECAUSE THE DATA THAT'S GENERATED IS AMONGST THE SAME TYPES OF PROJECTS. AND SO, THEREFORE, THE LEARNINGS ARE STRONGER; IS THAT RIGHT?

DR. PATEL: THAT'S CORRECT. AND THEY MAY ALSO PROVIDE MORE CLARITY TO THE APPLICANT AS TO WHAT IT MEANS TO HAVE A FOCUS AREA FOR THAT PROGRAM.

VICE CHAIR BONNEVILLE: OKAY, GREAT. SO THEN WHEN THIS COMES UP LATER, I THINK THAT'S DEFINITELY SOMETHING WE SHOULD CONSIDER. SO THANK YOU.

MR. TOCHER: I DON'T SEE ANY OTHER HANDS.

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1 CHAIRMAN FISCHER-COLBRIE: OTHER HANDS?
2 OKAY. GREAT. OKAY. LET'S GO ON TO THE NEXT AGENDA
3 ITEM. I THINK IT'S PROBABLY GIL.
4 DR. PATEL: THANK YOU. AND SO WE'RE GOING
5 TO SWITCH GEARS A LITTLE BIT NOW TO FOCUS ON
6 ENSURING THE GUIDING PRINCIPLES ARE APPLIED BY THE
7 RIGHT PARTY WITH THE RIGHT INFORMATION AT THE RIGHT
8 TIME. AND THIS IS A LOT OF WHAT PAT WAS TALKING
9 ABOUT, WHICH IS HOW DO WE TAKE THESE GUIDING
10 PRINCIPLES AND MAKE SURE THAT AT EVERY STEP OF THE
11 PROCESS THEY'RE RIGHTFULLY APPLIED WITH THE
12 APPROPRIATE INFORMATION AT HAND.
13 AND SO TO TEE UP THE REST OF THE
14 DISCUSSION, I'M GOING TO GO THROUGH WHAT OUR CURRENT
15 PROPOSAL IS AT EACH STEP, AND THEN GIL IS GOING TO
16 GET INTO THE DETAILS.
17 SO FIRST OF ALL, THE RESPONSIBLE PARTY.
18 SO FOR EACH STEP, VOLUME CONTROL, MERIT REVIEW, AND
19 FUNDING DECISION, WE'RE LAYING OUT WHO THE
20 APPROPRIATE PARTY WOULD BE. AND IN RED WE'RE
21 INDICATING WHAT HAS CHANGED FROM THE CURRENT
22 APPROACH.
23 SO FOR VOLUME CONTROL, AS WE NOTED, WE
24 PROPOSE TO HAVE THIS BE HANDLED BY THE GRANTS
25 WORKING GROUP. CURRENTLY, AS YOU KNOW, IT'S HANDLED

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1 BY THE CIRM TEAM WITH THE PREFERENCE-BASED APPROACH.
2 SO HERE THE RESPONSIBLE PARTY WILL BE THE GRANTS
3 WORKING GROUP MAKING THE SELECTIONS.
4 FOR MERIT REVIEW, IT HAS ALWAYS BEEN AND
5 WILL ALWAYS BE THE GRANTS WORKING GROUP'S
6 PREROGATIVE TO MAKE RECOMMENDATIONS BASED ON
7 SCIENTIFIC UNDERSTANDING OF THOSE APPLICATIONS.
8 AND THEN LASTLY, FOR THE FUNDING DECISION,
9 IT HAS ALWAYS BEEN AND WILL ALWAYS BE THE ARS'
10 PREROGATIVE TO MAKE FUNDING DECISIONS ON
11 APPLICATIONS IN ANY GIVEN CYCLE.
12 SO THE NEXT STEP OF THAT IS HOW OUR
13 GUIDING PRINCIPLES ARE BEING APPLIED. AND THEY'RE
14 BEING APPLIED TO THE CRITERIA OR THE FACTORS THAT
15 ARE GOING TO BE INCLUDED IN EACH OF THESE STEPS. SO
16 FOR THE VOLUME CONTROL, AT THE MOMENT WE HAVE
17 PREFERENCES. WE WOULD REPLACE THOSE SPECIFIC
18 SELECTION CRITERIA THE GRANTS WORKING GROUP WILL BE
19 EVALUATING AND MAKING SELECTIONS FOR APPLICATIONS
20 THAT WOULD MOVE ON TO SCIENTIFIC MERIT REVIEW. AND
21 GIL IS GOING TO DETAIL THE SELECTION CRITERIA FOR
22 EACH OF THE THREE PROGRAMS IN LATER SLIDES.
23 AT THE MERIT REVIEW STAGE, THERE ARE
24 EXISTING REVIEW CRITERIA FOR EACH OF THE PROGRAMS
25 THAT WERE BASED ON EXISTING GUIDING PRINCIPLES. AND

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1 THOSE WILL LARGELY REMAIN UNCHANGED BASED ON THE
2 BOARD FEEDBACK TO DATE.
3 AND LASTLY, ON THE FUNDING DECISION SIDE,
4 THERE ARE SEVERAL PROGRAMMATIC FACTORS THAT THE
5 BOARD CONSIDERS. WE'LL GET INTO THOSE LATER ON IN
6 THIS PRESENTATION, BUT NOT NECESSARILY ADDING OR
7 TAKING AWAY ANY OF THOSE FACTORS AT THIS POINT IN
8 THIS PROCESS.
9 AND LASTLY, WE'RE ADDRESSING THE POINT
10 ABOUT THE RIGHT INFORMATION. THIS IS WHERE CIRM'S
11 TEAM ROLE IS IS TO SUPPORT EACH OF THESE PROCESSES
12 BY BUILDING THE APPROPRIATE PROCESSES THEMSELVES BY
13 DEFINING THE SELECTION CRITERIA AND PROVIDING THE
14 APPROPRIATE LEVEL OF INFORMATION TO EACH PARTY TO
15 MAKE ANY SELECTIONS, RECOMMENDATIONS, OR DECISIONS.
16 SO TO GIVE YOU AN EXAMPLE ON THE VOLUME
17 CONTROL SIDE, IT'S TO DEFINE THOSE SELECTION
18 CRITERIA, THE GWG COMPOSITION, AS WELL AS THE
19 PROCESS ITSELF IN TERMS OF THE ASSIGNMENTS, THE
20 SCORING, THE FEEDBACK.
21 SWITCHING THEN OVER TO THE FUNDING
22 DECISION SIDE, OUR PROPOSAL IS TO PROVIDE A MORE
23 STRUCTURED INFORMATION PACKAGE TO THE BOARD TO
24 INFORM YOUR DECISION-MAKING IN APPROVING THOSE
25 AWARDS. AND SO THIS WOULD INCLUDE, AS IT ALWAYS

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1 DOES, THE GRANTS WORKING GROUP RECOMMENDATION, BUT
2 ADDING A COUPLE MORE ITEMS AS TO HOW EACH OF THOSE
3 APPLICATIONS IS RESPONSIVE TO THE GUIDING PRINCIPLES
4 AS WELL AS A MORE STRUCTURED APPROACH TO OUR OWN
5 CIRM TEAM FUNDING RECOMMENDATION. AND I'LL GET BACK
6 TO THOSE POINTS AFTER GIL'S PRESENTATION.
7 SO AT THIS POINT WE'RE GOING TO TRANSITION
8 TO GIL TO WALK THROUGH THE PROCESSES PARTICULARLY
9 FOR VOLUME CONTROL.
10 DR. SAMBRANO: ALL RIGHT. THANK YOU,
11 SHYAM. YOU CAN GO AHEAD AND GO TO THE NEXT SLIDE.
12 SO AS SHYAM INDICATED, THE VOLUME CONTROL STEP IS
13 SOMETHING THAT IS VERY IMPORTANT AND OVER TIME AND
14 PARTICULARLY NOW WITH THE INCREASE IN OVERALL DEMAND
15 WITH OUR APPLICATIONS, IT'S A NECESSARY STEP IN OUR
16 PROCESS.
17 IN THE PAST WE HAVE USED DIFFERENT
18 MECHANISMS. WE'VE USED A PREAPPLICATION PROCESS.
19 WE'VE EVEN LIMITED THE TOTAL NUMBER OF APPLICATIONS
20 THAT COULD BE SUBMITTED BY A GIVEN INSTITUTION. AND
21 THEN OBVIOUSLY WE'VE ALSO TRIED THE PREFERENCES.
22 ALL HAVE HAD SOME BENEFITS TO THEM AND BEEN
23 EFFECTIVE IN NARROWING THE POOL, BUT THERE ARE
24 CONSEQUENCES TO EACH THAT I THINK ARE IMPORTANT TO
25 CONSIDER AND TO LEARN FROM.

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1 AND SO WHAT WE'RE GOING TO TALK ABOUT IS
2 WHAT WE'RE PROPOSING TO DO WITH VOLUME CONTROL
3 MOVING FORWARD THAT WE THINK ADDRESS A LOT OF THE
4 CONCERNS AND THE ELEMENTS THAT WE THOUGHT WERE
5 LACKING IN THE PREFERENCE APPROACH.
6 SO THE GOAL, OF COURSE, IS TO NARROW THE
7 POOL. AND ULTIMATELY WHAT WE WANT TO DO WITH
8 WHATEVER METHOD WE CHOOSE IS TO REMOVE WHAT CLEARLY
9 HAS NO MERIT AMONG A POOL OF APPLICATIONS TO ENRICH
10 THEM FOR THOSE THAT ARE BEST ALIGNED WITH OUR GOALS
11 AND TO HAVE A MORE RIGOROUS REVIEW OF THOSE THAT ARE
12 MOST COMPETITIVE. NEXT SLIDE PLEASE.
13 SO I WANT TO SHOW YOU THIS TABLE TO GIVE
14 YOU A SENSE OF THE EXTENT OF THE VOLUME CONTROL THAT
15 WE NEED ACROSS EACH OF THE DIFFERENT PROGRAMS. NOW,
16 THIS IS SORT OF INTENDED TO BE A LITTLE BIT OF A
17 COMPARATOR. SO EVERYTHING IS BASED ON IF ALL OF
18 THESE WOULD REQUIRE A FULL APPLICATION; BUT AS
19 YOU'LL NOTE LATER, FOR THE DISC4 WE WON'T BE ASKING
20 FOR THAT, BUT JUST TO GIVE YOU A SENSE.
21 SO THERE'S THE TARGET FOR MERIT REVIEW.
22 THAT'S WHAT WE BELIEVE IS THE MOST APPROPRIATE FOR A
23 RIGOROUS QUALITY REVIEW FOR EACH OF THESE PROGRAMS.
24 SO THEY CAN RANGE FROM UP TO TEN TO ABOUT 50
25 DEPENDING ON THE PROGRAM. AND THEN IN THE THIRD ROW

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1 IS THE NUMBER OF AWARDS THAT WE EXPECT TO ISSUE PER
2 CYCLE. SO THOSE THAT ARE REVIEWED LEAD YOU TO ABOUT
3 THIS MANY THAT WE WOULD WANT TO TARGET. OFTENTIMES
4 EVEN WITH THESE NUMBERS, WE END UP WITH MORE
5 RECOMMENDED THAN WE CAN FUND FOR ANY OF THESE
6 PROGRAMS. AND THEN THE LAST ROW IS JUST THE CYCLES
7 PER YEAR THAT WE HAVE.
8 ONE IMPORTANT NOTE AS IT RELATES TO THIS
9 TABLE IS THAT IF DEMAND CHANGES FOR ANY OF THESE
10 PROGRAMS, THAT CAN HELP ADJUST. IF IT WERE TO
11 LOWER, THEN WE CAN DO SOMETHING DIFFERENT IN TERMS
12 OF VOLUME CONTROL. BUT IF IT INCREASES, WE STILL
13 CAN ONLY FUND SO MANY APPLICATIONS PER CYCLE PER
14 YEAR DEPENDING ON HOW WE'VE LAID THAT OUT IN THE
15 BUDGET.
16 SO NEXT SLIDE PLEASE. SO THE GOALS OF OUR
17 PROPOSED PROCESS WAS TO REFOCUS THE SELECTION OF
18 APPLICATIONS ON THE SCIENCE RATHER THAN ON STRICT
19 PREFERENCES FOR ALL THE REASONS THAT WE'VE ALREADY
20 NOTED. WE IDEALLY WOULD WANT TO ALIGN THE PROGRAMS
21 WITH A MORE CONSISTENT VOLUME CONTROL PROCESS ACROSS
22 THE DIFFERENT PROGRAMS BECAUSE THIS WOULD CERTAINLY
23 HELP WITH APPLICANTS BETTER UNDERSTANDING WHAT'S
24 HAPPENING FOR US TO COMMUNICATE AND USE IT TO APPLY
25 WITH THIS MINIMAL VARIABILITY.

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AND WITH WHAT WE WANT TO PROPOSE, WE CAN ALSO LEVERAGE THE LONG-STANDING LEARNINGS WE'VE HAD FROM OUR POSITIVE SELECTION PROCESS THAT WAS IMPLEMENTED SINCE 2015 TO CONTINUE TO MAKE IMPROVEMENTS. AND I'LL TELL YOU ABOUT HOW WE BELIEVE WE CAN IMPROVE IT QUITE A BIT IN THE NEW PROPOSED METHOD.

WE ALSO WERE LOOKING TO INCREASE EFFICIENCY IN THE OPPORTUNITY TO LEARN AND IMPLEMENT IMPROVEMENTS ACROSS THE PROGRAMS IF THEY ARE MORE UNIFORM. NEXT SLIDE.

SO THIS IS THE TABLE THAT SHOWS WHAT OUR CURRENT VOLUME CONTROL PROCESSES ARE ACROSS THESE FOUR PROGRAMS OF DISC5, DISC4, PDEV, AND CLIN2. AND AS YOU WILL NOTICE, WE HAVE VERY DIFFERENT APPROACHES. WE HAVE IN ONE CASE POSITIVE SELECTION, IN A COUPLE OF THEM A RESUBMISSION PROCESS WHERE THE PREFERENCES WERE APPLIED BY CIRM STAFF, AND THEN CLIN2, WHICH IS A MODIFICATION OF THAT WHICH WE CALL A QUALIFICATION PROCESS THAT ALSO MAKES USE OF PREFERENCES. AND THE VARIABILITY IS ALSO IN WHO IS MAKING THESE SELECTIONS.

SO FOR DISC5, FOR EXAMPLE, YOU SEE IT IS THE GRANTS WORKING GROUP DO THE POSITIVE SELECTION PROCESS. IN OTHERS IT IS THE CIRM STAFF OR A

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1 COMBINATION OF CIRM AND GRANTS WORKING GROUP. SO
2 QUITE A BIT OF VARIABILITY IN TERMS OF WHAT IS DONE
3 AS WELL AS THE BASIS FOR SELECTION. NEXT SLIDE.
4 AND THIS IS WHAT WE PROPOSE AND WHAT I
5 WILL GIVE YOU MORE DETAILS ABOUT AS WE MOVE FORWARD.
6 WE PROPOSE TO HAVE A PROCESS, A MORE UNIFIED
7 PROCESS, THAT WE REFER TO ASSESS GRANTS WORKING
8 GROUP SELECTION. IT IS BASED ON THE EXISTING
9 POSITIVE SELECTION, BUT IT HAS IMPORTANT
10 DIFFERENCES. THIS MISSION IN TERMS OF WHAT WE WOULD
11 EXPECT FROM THE APPLICANTS COMING IN IS IN MOST
12 CASES A FULL APPLICATION. AND FOR DISC4 WE ARE
13 REQUESTING A PRESUBMISSION FORM OR A SHORT-FORM
14 APPLICATION.
15 THE SELECTION IN ALL CASES IS CONSISTENT.
16 IT'S ALL BY GRANTS WORKING GROUP. AND THE BASIS FOR
17 SELECTION FOR ALL OF THESE IS SCIENTIFIC VALUE. SO,
18 AGAIN, TO REITERATE, FOCUSING ON SCIENCE, MAKING IT
19 MORE CONSISTENT AND MORE EFFICIENT AS WE MOVE
20 FORWARD. NEXT SLIDE PLEASE.
21 AND SO JUST A WARNING. THESE NEXT THREE
22 SLIDES ARE SLIDES THAT WERE ADDED INTO THE DECK. SO
23 THOSE WEREN'T POSTED, BUT WE WILL POST THESE
24 FOLLOWING THIS MEETING. WE ADDED THESE SLIDES JUST
25 TO HELP US IN TERMS OF PRESENTATION CLARITY.

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1 SO WE HAVE MOVING FORWARD WHAT WE BELIEVE
2 IS A MORE UNIFIED GRANTS WORKING GROUP SELECTION
3 PROCESS. I WILL TALK TO YOU ABOUT THESE ELEMENTS
4 THAT ARE BULLETED. THE PROCESS ESTABLISHES A TARGET
5 NUMBER OF APPLICATIONS FOR FULL PANEL DISCUSSION FOR
6 EACH PROGRAM. IT SPECIFIES WHAT WOULD BE THE
7 OPTIMAL AND FIXED NUMBER OF REVIEWS THAT OCCUR PER
8 APPLICATION, THE NUMBERS OF APPLICATIONS REVIEWED BY
9 EACH REVIEWER, AND THE TOTAL NUMBER OF REVIEWERS
10 THAT PARTICIPATE IN EACH. GRANTS WORKING GROUP
11 MEMBERS UNDER THIS PROCESS EVALUATE APPLICATIONS
12 BASED, AGAIN, ON SCIENTIFIC VALUE THAT'S DESCRIBED
13 IN THE RESPECTIVE PROGRAM ANNOUNCEMENT AND THAT
14 ALIGN WITH GUIDING PRINCIPLES. AND WE'LL GO OVER
15 THAT. AND THEN APPLICATIONS RECEIVE A COMPOSITE
16 SCORE THAT INCLUDES RANKING AS ONE OF THE MEASURES,
17 AND I WILL GO OVER THAT. AND ADVANCEMENT OF THOSE
18 APPLICATIONS IS DETERMINED BY THE COMPOSITE SCORE
19 WITH A CUTOFF MADE AT A NATURAL BREAK NEAREST THE
20 TARGET. THERE IS THE ADDITION OF HAVING MORE
21 APPLICATIONS WITH A LIMIT OF UP TO THREE THAT CAN BE
22 ADVANCED BY THE CIRM PRESIDENT. NEXT SLIDE.
23 SO THIS TABLE HIGHLIGHTS SOME OF THE
24 DIFFERENCES VIA EXAMPLE OF THE EFFECT OF DIFFERENT
25 NUMBERS OF APPLICATIONS THAT CAN COME IN IN AN

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1 APPLICATION POOL AND HOW WE CAN MANIPULATE THE
2 NUMBER OF APPLICATIONS PER REVIEWER, THE NUMBER OF
3 REVIEWS PER APPLICATION IN ORDER TO GET THE TOTAL
4 NUMBER OF REVIEWS THAT WE NEED FOR THAT GIVEN CYCLE
5 AND THAT HELP US TARGET FOR ADVANCEMENT THE
6 APPROPRIATE NUMBER OF APPLICATIONS.
7 SO IN THESE SET OF EXAMPLES, WE HAVE AN
8 APPLICATION POOL THAT FOR DISC5 ON ONE EXTREME IS
9 ABOUT 200 APPLICATIONS, FOR CLIN2 ON THE OTHER
10 EXTREME IS ONLY ABOUT 20. THE TOTAL NUMBER OF
11 REVIEWERS THAT WOULD PARTICIPATE IN THIS, WE'RE JUST
12 HOLDING STEADY FOR THE SAKE OF THIS EXAMPLE AT 20.
13 APPLICATIONS FOR REVIEWERS BASED ON THAT, MEANING
14 THAT'S THE SIZE OF THE BATCH THAT EACH REVIEWER
15 WOULD LOOK THROUGH AND WOULD REVIEW SPECIFICALLY IN
16 ORDER TO DETERMINE WHAT THEY FEEL ARE THE MOST
17 MERITORIOUS BASED ON THE CRITERIA THAT WE WILL
18 DISCUSS.
19 SO FOR DISC5, EACH REVIEWER MAY BE LOOKING
20 AT ABOUT 30 APPLICATIONS; WHEREAS, AT CLIN2, THEY
21 MAY ONLY BE LOOKING AT FIVE IN ORDER TO GET THE
22 APPROPRIATE COVERAGE. BUT GIVEN THE NUMBER OF
23 APPLICATIONS, THAT MEANS THAT THE NUMBER OF REVIEWS
24 PER APPLICATION WITH A VERY LARGE NUMBER IS GOING TO
25 BE A LITTLE LESS. SO MAYBE THREE REVIEWS PER

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1 APPLICATION AT THE DISC5 STAGE WHERE WE CAN MORE
2 EASILY DO ABOUT FIVE PER APPLICATION AT THE CLIN2
3 STAGE. AND I'LL NOTE THAT THESE WE CONSIDER TO BE
4 MINIMUMS, MEANING THAT WE CAN ADJUST THESE NUMBERS
5 SO THAT THE NUMBER OF REVIEWS PER APPLICATION EVEN
6 AT DISC5 COULD INCREASE TO FOUR. BUT WE WOULD MAKE
7 IT SO THAT IT'S FIXED, MEANING EVERY APPLICATION
8 WOULD EITHER GET THREE OR FOUR DEPENDING ON THE
9 PARAMETERS.
10 MR. TOCHER: HEY, GIL?
11 DR. SAMBRANO: YES.
12 MR. TOCHER: MARIA HAS HER HAND UP.
13 DR. SAMBRANO: OH, I'M SORRY. MARIA, GO
14 AHEAD.
15 VICE CHAIR BONNEVILLE: THAT'S OKAY. I
16 JUST NOTED THERE'S 20 REVIEWERS, BUT THERE ARE 15
17 SCIENTIFIC AND 7 PATIENT ADVOCATES. SO THAT ALREADY
18 IS MORE THAN 20. SO WHAT'S THE PATIENT ADVOCATE
19 RESPONSIBILITY? HOW ARE YOU FACTORING THAT IN?
20 DR. SAMBRANO: SO FOR THIS STEP, AND SAME
21 AS WHAT WE'VE DONE WITH POSITIVE SELECTION, WE'VE
22 USED MEMBERS OF THE GRANTS WORKING GROUP, BUT WE
23 HAVE IT LIMITED TO 15 BECAUSE THEY'RE NOT ACTING AS
24 A PANEL. THEY ARE ACTING INDIVIDUALLY LOOKING AT
25 BATCHES OF APPLICATIONS RATHER THAN AS A SINGLE

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1 SCORING VOTING PANEL THAT WE TYPICALLY HAVE. AND
2 SO --
3 VICE CHAIR BONNEVILLE: I DIDN'T
4 UNDERSTAND THAT. I'M SORRY.
5 DR. SAMBRANO: OKAY. I'M NOT SURE HOW
6 ELSE TO EXPLAIN. I MEAN --
7 VICE CHAIR BONNEVILLE: WELL, RIGHT NOW,
8 FOR EXAMPLE, I WAS ASKED TO GO IN TO POSITIVE
9 SELECTION AND CHOOSE SOME APPLICATIONS. SO IT ASKED
10 ME AS A PANELIST TO CHOOSE SOME AS PART OF THE GWG
11 REVIEW. I'M SURE CHRIS GOT THE EMAIL, ET CETERA.
12 SO I'M WONDERING HOW DOES THAT FACTOR INTO THE TOTAL
13 NUMBER OF REVIEWERS? IS IT AN EQUIVALENCE? IS IT
14 DIFFERENT? HOW IS THAT DETERMINED?
15 DR. SAMBRANO: RIGHT. SO THE NUMBER OF
16 REVIEWERS IS DETERMINED BY WHAT ALLOWS US TO HAVE
17 SUFFICIENT REVIEWERS TO GET COVERAGE, SUFFICIENT
18 COVERAGE, AND OVERLAP OF APPLICATIONS FOR EQUIVALENT
19 BATCH SIZE FOR ALL REVIEWERS. AND SO THESE CAN BE
20 OPTIMIZED IN ORDER TO ACHIEVE THAT NUMBER. AND SO
21 20 IN THIS EXAMPLE IS A NUMBER THAT HELPS BALANCE
22 THE COVERAGE THAT WE CAN GET WHILE AT THE SAME TIME
23 ENSURING THAT EACH APPLICATION, THE BATCH THAT A
24 GROUP HAS IS AS REPRESENTATIVE OF THE LARGER POOL AS
25 POSSIBLE.

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1 BUT TO YOUR QUESTION OF HOW DO WE EXCEED
2 THE 15, THIS IS SOMETHING THAT WE EXPLORED WITH
3 LEGAL IN TERMS OF BEING ABLE TO DO THIS AT THIS
4 STAGE. WE'VE DONE IT FOR THE POSITIVE SELECTION
5 PROCESS A FEW TIMES. WE'VE INCREASED THE NUMBER SO
6 THAT WE COULD HAVE MORE EXPERTISE BROUGHT TO THE
7 TABLE IN LOOKING AT APPLICATIONS AT THIS STAGE. AND
8 SO THIS REPRESENTS THE NUMBER OF SCIENTIFIC
9 REVIEWERS.
10 NOW, THE PATIENT ADVOCATE MEMBERS, AS YOU
11 MENTIONED UNDER --
12 VICE CHAIR BONNEVILLE: THAT'S WHAT I
13 MEANT. SORRY. I KNOW THIS REPRESENTS THE
14 SCIENTIFIC MEMBERS. MY SPECIFIC QUESTION IS HOW
15 DOES THIS AFFECT THE PATIENT ADVOCATES?
16 DR. SAMBRANO: RIGHT. SO THE PATIENT
17 ADVOCATE MEMBERS UNDER THE POSITIVE SELECTION
18 PROCESS FUNCTIONED IN MANY WAYS JUST LIKE THE GRANTS
19 WORKING GROUP REVIEWERS. THEY WERE ASKED TO PICK
20 ANY APPLICATION. IF THEY PICKED IT, THE APPLICATION
21 ADVANCED. WE'RE MOVING AWAY FROM THAT MODEL. AND
22 SO IN THIS INSTANCE, THE PATIENT ADVOCATES ARE NOT
23 PART OF THIS CALCULATION. THIS IS ONLY THE
24 SCIENTIFIC MEMBERS.
25 HOW THE PATIENT ADVOCATE MEMBERS COME INTO

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1 THIS STEP IS SOMETHING THAT STILL NEEDS TO BE
2 CONSIDERED. WE'D LIKE TO OVERLAY IT ON THIS RATHER
3 THAN IT BE THE SAME THING. IN OTHER WORDS, RATHER
4 THAN ASSIGNING A PATIENT ADVOCATE MEMBER A BATCH OF,
5 SAY, 30 APPLICATIONS TO HAVE THE SAME ASSIGNMENT AS
6 THE SCIENTIFIC MEMBERS, MAY NOT BE APPROPRIATE. SO
7 THAT IS SOMETHING THAT WE WANT TO CONTINUE TO
8 DISCUSS.
9 VICE CHAIR BONNEVILLE: THANK YOU.
10 DR. SAMBRANO: ALL RIGHT. NEXT SLIDE
11 PLEASE.
12 CHAIRMAN IMBASCIANI: GIL?
13 DR. SAMBRANO: YES.
14 CHAIRMAN IMBASCIANI: THIS IS VITO.
15 BEFORE YOU MOVE ON, I JUST WANT TO LOOK AT THAT
16 CHART FROM A DIFFERENT PERSPECTIVE, A STATISTICAL
17 PERSPECTIVE. MANY OF OUR BOARD MEMBERS AND ALL OF
18 OUR PRINCIPAL INVESTIGATORS LIVE IN A WORLD OF
19 GAUSSIAN DISTRIBUTIONS, AND THEY'RE CONCERNED ABOUT
20 PROBABILITIES, THE POWER OF SAMPLE POPULATIONS,
21 PRECISION, PREDICTIVE VALUE. SO I'M VERY
22 COMFORTABLE IF THIS NUMBER WAS JUST ARRIVED AT 20
23 REVIEWERS, EACH DOING 30 APPLICATIONS, COMING UP
24 WITH A SAMPLE WITH A POT THAT YOU'RE GOING TO WORK
25 WITH OF 600. BUT HAVE YOU TRIED ANY STATISTICAL

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1 APPLICATION THAT MIGHT LEND SOME AUTHORITY TO THAT?
2 DR. SAMBRANO: YES. SO ONE OF THE THINGS
3 THAT WE HAVE BEEN DOING IS RUNNING SIMULATIONS OF
4 DIFFERENT MODELS AND APPROACHES. ONE OF THE THINGS
5 I WAS GOING TO MENTION IN DISCUSSING THE COMPOSITE
6 SCORE AND THE FLOWCHART THAT I'M GOING TO SHOW YOU
7 IN A MINUTE IS THAT WE'VE RUN SIMULATIONS USING
8 DIFFERENT METHODS. AND A METHOD THAT USES BOTH
9 SELECTION AND RANK PERFORMS BETTER THAN OTHERS
10 BECAUSE IT PROVIDES THE BEST CUTOFF CLARITY AMONG
11 ALL THE DIFFERENT ONES THAT WE TESTED.
12 THE METHOD ALSO IS VERY GOOD AT CORRECTLY
13 ADVANCING APPLICATIONS THAT WE WOULD EXPECT TO SCORE
14 85 OR ABOVE. AND SO THIS IS BASED ON DATA THAT WE
15 HAVE FROM EXISTING APPLICATIONS OR THE HISTORY OF
16 APPLICATIONS THAT WE'VE HAD.
17 SO THE CATCH RATE, IF YOU WILL, DEPENDING
18 ON THE VOLUME, WITH THE HIGHEST VOLUMES BEING THE
19 ONES THAT PROVIDE THE LEAST CERTAINTY, IT WILL RANGE
20 FROM 77 TO 100 PERCENT USING THIS APPROACH. IN
21 TERMS OF ITS ABILITY TO REMOVE NONCOMPETITIVE
22 APPLICATIONS, IT RANGES FROM ABOUT 73 TO 99 PERCENT.
23 AND SO THAT IS LOOKING AT RANGES THAT COVER IN THE
24 SEVERAL HUNDRED DOWN TO ABOUT THE 20 THAT YOU SEE IN
25 CLIN2.

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1 SO IN COMPARING THESE, YES, THERE ARE
2 ADVANTAGES TO THAT THAT WE'VE BEEN EXPLORING USING
3 REAL DATA AND ALSO JUST STATISTICALLY LOOKING AT AND
4 MATCHING UP WITH THE APPROPRIATE BATCH AMOUNTS WOULD
5 BE PER REVIEWER AND WHAT HAPPENS IF YOU EITHER
6 INCREASE THE NUMBER OF REVIEWERS OR DECREASE THE
7 NUMBER OF REVIEWERS.
8 ONE OF THE THINGS THAT WE LEARNED, WE
9 ASSUMED SOMETIMES THAT BY INCREASING THE NUMBER OF
10 REVIEWERS, THAT WOULD ALWAYS BE BETTER, BUT THAT'S
11 NOT ACTUALLY THE CASE BECAUSE ONE OF THE THINGS THAT
12 HAPPENS IS, IF YOU HAVE TOO MANY REVIEWERS, YOU MAY
13 BENEFIT FROM HAVING A LOT OF DIFFERENT EXPERTISE,
14 BUT THEN THE SAMPLING FROM THE LARGER POOL IS MUCH
15 LESS PER REVIEWER. SO THEY HAVE LESS OF A SENSE OF
16 THE OVERALL POOL THAN A SMALLER NUMBER WOULD.
17 AND SO IT'S TRYING TO BALANCE THESE
18 DIFFERENT ELEMENTS TO GIVE YOU WHAT MAY BE, IF YOU
19 WILL, A SWEET SPOT FOR EACH OF THESE DIFFERENT
20 PROGRAMS DEPENDING ON THE EXPECTED POOLS THAT YOU
21 WOULD HAVE FOR THAT PROGRAM.
22 MR. TOCHER: MARK HAS HIS HAND RAISED.
23 DR. SAMBRANO: MARK.
24 CHAIRMAN FISCHER-COLBRIE: HEY, GIL, AS A
25 PATIENT ADVOCATE, I'M SEEING IN MANY CASES THE

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1 PATIENT ADVOCATES PROVIDE INCREDIBLY UNIQUE VANTAGE
2 POINTS AROUND APPLICATIONS FROM A PERSPECTIVE THAT
3 OTHERS MIGHT MISS. AND HISTORICALLY, OBVIOUSLY,
4 THEY'VE BEEN PART OF THE POSITIVE SELECTION PROCESS.
5 I'D LIKE TO UNDERSTAND A LITTLE BIT MORE ABOUT WHY
6 PATIENT ADVOCATES' ROLE IN THIS CONTEXT ARE REMOVED
7 AND WHY THAT'S NECESSARY AS PART OF THIS PROCESS.
8 DR. SAMBRANO: RIGHT. SO PART OF THE
9 PROCESS -- AND I'M TRYING TO REMEMBER IF I HAVE A
10 SLIDE ON IT. I THINK LATER, BUT I CAN TELL YOU NOW.
11 PART OF THE PROCESS INVOLVES DOING EXPERTISE MATCH
12 ASSIGNMENTS WITH APPLICATIONS. SO ALL OF THE
13 REVIEWERS NOTED IN THIS LAYOUT ARE GOING TO HAVE THE
14 BATCH OF THE APPLICATIONS GENERALLY MATCHED TO THAT
15 EXPERTISE. AND SO THAT IS IN PART WHAT REMOVES THE
16 PATIENT ADVOCATE MEMBERS FROM IT.
17 ON THE OTHER HAND, AS YOU NOTED, THERE IS
18 GREAT VALUE IN HAVING PATIENT ADVOCATE PERSPECTIVE.
19 SO WHAT WE WANT TO PROPOSE IS THAT, RATHER THAN
20 REMOVING PATIENT ADVOCATES IN THE STRAIGHT-UP
21 EXPERTISE MATCH, THAT WE HAVE A DIFFERENT LAYER OF
22 PATIENT ADVOCATE INVOLVEMENT AT THIS STEP. BUT AT
23 THIS TIME I DON'T HAVE A CLEAR SOLUTION.
24 CHAIRMAN FISCHER-COLBRIE: I THINK WE NEED
25 TO ADDRESS THIS BECAUSE, RELATED TO EXPERTISE, FOR

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1 EXAMPLE, THERE ARE PEOPLE WHO HAVE LONG HISTORIES
2 WITH THE PARKINSON'S GROUP OR ALS OR TYPE 1 DIABETES
3 IN MY CASE AND THINGS LIKE THAT. AND I THINK IT'S
4 IMPORTANT BECAUSE THEY CAN, IN FACT, BRING A
5 DIFFERENT KIND OF EXPERTISE, IF YOU WILL, OR
6 PERSPECTIVES THAT I THINK IS INHERENT FOR WHY EVEN A
7 PATIENT ADVOCATE ROLE WAS CREATED TO BEGIN WITH.
8 SO I JUST WANT TO MAKE SURE THAT WE NOODLE
9 THROUGH THIS AND MAKE SURE WE UNDERSTAND EXACTLY
10 WHAT THE CYCLE IS THAT IS INVOLVED. AND IF THERE IS
11 A CHANGE, BE ABLE TO EXPLAIN TO PEOPLE WHY THEY'RE
12 NO LONGER PART OF THE PROCESS.
13 DR. SAMBRANO: RIGHT. OKAY. THAT MAKES
14 SENSE, MARK. THANK YOU.
15 DR. YAMAMOTO: GIL, I'M CONFUSED. I NEED
16 YOU TO HELP ME OUT HERE. I WAS GOING TO RAISE THIS
17 LATER, BUT IT'S ACTUALLY EASY TO DO IN THE CONTEXT
18 OF THIS FIGURE. SO MY UNDERSTANDING OF VOLUME
19 CONTROL IN THE PREVIOUS SYSTEM, THE PREFERENCE CHECK
20 BOX SYSTEM, WAS TO LIMIT THE VOLUME OF GRANT
21 APPLICATIONS THAT WERE REVIEWED. WE SAW THE
22 PROBLEMS WITH THAT, BUT I THINK THAT WAS THE GOAL.
23 AND HERE IT LOOKS LIKE EVERYTHING GETS REVIEWED, AND
24 THE VOLUME CONTROL IS IN TERMS OF THE NUMBER OF
25 APPLICATIONS THAT WILL GO FORWARD FOR DISCUSSION BY

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1 THE GWG. SO DO I HAVE THAT RIGHT? AND IF SO, IS
2 THAT THE APPROPRIATE PLACE FOR VOLUME CONTROL TO BE
3 EXERTED?
4 ONE COULD ARGUE, AND WE HAVE ARGUED
5 PREVIOUSLY, THAT IT'S IMPORTANT TO HAVE SOME KIND OF
6 CONTROL ON THE NUMBER OF APPLICATIONS THAT ARE
7 REVIEWED BECAUSE AS THAT NUMBER GETS OUT OF CONTROL,
8 THEN THE QUALITY OF THE REVIEWS GETS AT LEAST
9 THREATENED IF NOT ERODED BECAUSE OF JUST EXHAUSTION
10 OF THE REVIEWERS. YOU'RE ASKING A LOT OF THEM.
11 AND BUT WHAT I'M SEEING IN THIS APPROACH
12 IS THAT EVERYTHING THAT COMES IN WILL BE REVIEWED,
13 AND THEN THERE WILL BE VOLUME CONTROL EXERTED AT THE
14 LEVEL OF THE NUMBER OF REVIEWED APPLICATIONS THAT
15 ARE THEN DISCUSSED BY GWG. SO DO I HAVE THAT RIGHT?
16 AND IS THERE -- HAVE THOSE VERY IMPORTANT
17 DIFFERENCES BETWEEN THE TWO APPROACHES BEEN
18 DISCUSSED?
19 DR. SAMBRANO: RIGHT. SO IT'S A LITTLE
20 DIFFERENT THAN WHAT YOU DESCRIBE IN THE SENSE THAT
21 EVERY APPLICATION DOESN'T GET A FULL REVIEW. SO
22 THIS IS MORE ANALOGOUS TO OUR POSITIVE SELECTION
23 PROCESS WHERE WHAT EACH REVIEWER RECEIVES IS A BATCH
24 OF APPLICATIONS, SO AN EXAMPLE OF, SAY, 30, AND THEY
25 ARE ASKED TO SELECT THE FIVE THAT THEY FEEL ARE THE

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1 MOST MERITORIOUS, BUT BASED ON A LIMITED CRITERION.
2 AND I WILL GO OVER WHAT THAT IS. IT'S NOT THE FULL
3 SET OF CRITERIA THAT THEY WOULD DO IN A FULL REVIEW.
4 THIS IS THE HIGH LEVEL SCIENTIFIC VALUE OF THIS
5 PROPOSAL EQUIVALENT TO SIGNIFICANCE, IMPACT, IF YOU
6 WILL, OF A SET OF CRITERIA.
7 SO THEY SELECT BASED ON THAT, AND THEY
8 SELECT THEIR FIVE BEST OUT OF THE BATCH OF 30 AND
9 THEY RANK THEM. AND SO THEY RANK EACH OF THOSE 1
10 THROUGH 5. AND SO WHEN WE SAY REVIEWS PER
11 APPLICATION, WE MEAN THAT EACH APPLICATION IS GOING
12 TO GET THREE REVIEWS FROM THREE DIFFERENT REVIEWERS.
13 IT'S GOING TO BE IN THREE DIFFERENT BATCHES SO IT
14 HAS THREE OPPORTUNITIES IN THAT EXAMPLE TO BE
15 SELECTED TO ADVANCE. AND IF IT'S SELECTED TO
16 ADVANCE, IF IT MEETS THE CUTOFF, THEN IT GOES TO A
17 FULL PANEL REVIEW, WHICH MEANS THEN WE ASSIGN
18 SPECIFIC EXPERTS IN THAT APPLICATION AND WE DESIGN
19 THE PANEL AROUND THE APPLICATIONS THAT ARE SELECTED
20 TO HAVE A FULL, ROBUST DISCUSSION OF EACH OF THE
21 APPLICATIONS THAT ADVANCE, DO OUR TYPICAL 1 TO 100
22 SCORING, A FULL CRITIQUE OF THOSE APPLICATIONS, AND
23 PROVIDE A FULL SUMMARY OF APPLICANTS. AND SO THAT'S
24 THE DISTINCTION BETWEEN THOSE TWO STEPS.
25 DR. PATEL: IF I MAY JUST ADD A

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1 CLARIFICATION. SO GIL IS DESCRIBING THE VOLUME
2 CONTROL STEP HERE. SO THIS IS STILL THE VOLUME
3 CONTROL STEP, BUT NOT NECESSARILY A TWO-STEP MERIT
4 REVIEW YET.
5 DR. SAMBRANO: CORRECT.
6 DR. YAMAMOTO: SO I GUESS FOR ME, THEN, A
7 LOT WOULD DEPEND ON WHAT ARE THE CRITERIA THAT ARE
8 USED IN THIS INITIAL REVIEW? I DON'T KNOW WHAT ELSE
9 TO CALL IT OTHER THAN REVIEW. BUT IN THIS INITIAL
10 STEP, THAT DIFFERS FROM REVIEWING -- SO THE
11 APPLICANTS -- I'M SORRY -- THE REVIEWERS GET THE
12 FULL APPLICATION, BUT THEN THEY'RE ASKED TO EXAMINE
13 A SMALLER NUMBER OF THINGS RATHER THAN A FULL
14 REVIEW. DO I HAVE THAT RIGHT? AND THEN WHAT ARE
15 THOSE CRITERIA?
16 DR. SAMBRANO: IT DEPENDS ON THE PROGRAM.
17 SO FOR DISC4, WE'RE NOT ASKING FOR A FULL
18 APPLICATION. FOR PDEV AND DISC5 WE ARE. THE DISC5
19 IS, RELATIVELY SPEAKING, A SHORTER APPLICATION. AND
20 THE VOLUME IS SO HIGH. ONE OF THESE, FOR THESE
21 TYPES OF PROGRAMS, WE EASILY GOT OVER 300 IN THE
22 DISC-0, WHICH WAS THE PREVIOUS VERSION OF DISC5.
23 THAT WAS A FULL APPLICATION. SO THE VOLUME IS
24 EXTREMELY HIGH THERE.
25 FOR THE PDEV AND CLIN2, ONE OF THE VERY

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1 IMPORTANT THINGS THERE IS THE READINESS ELEMENT AND
2 ENSURING THAT THESE PROGRAMS CAN ADVANCE QUICKLY.
3 AND SO FOR THAT, THE FULL APPLICATION ACTUALLY IS
4 QUITE IMPORTANT FOR US IN ORDER TO UNDERSTAND THAT
5 THESE PROGRAMS ARE READY AND AT THE APPROPRIATE
6 STAGE TO ADVANCE.
7 BUT AS MENTIONED FOR THE DISC4, WE ARE, I
8 THINK BECAUSE OF THE WAY THAT'S STRUCTURED, WE CAN
9 ALLOW A LESS THAN FULL APPLICATION AT THAT STAGE.
10 THE CONSEQUENCE THOUGH IS THAT YOU END UP WITH A
11 MUCH LONGER REVIEW CYCLE BECAUSE YOU HAVE TO GO
12 THROUGH THIS PROCESS OF DETERMINING WHICH ONES
13 ULTIMATELY ARE SELECTED TO HAVE A FULL APPLICATION,
14 AND YOU HAVE TO GIVE AT LEAST A COUPLE OF MONTHS FOR
15 THAT APPLICATION TO BE COMPLETED BY THE APPLICANTS
16 BEFORE YOU CAN PROCEED. SO IT DOES EXTEND THE LIFE
17 CYCLE.
18 DR. YAMAMOTO: OKAY. SO I CAN UNDERSTAND
19 FOR THE SITUATIONS IN WHICH THERE'S NOT A FULL
20 APPLICATION. BUT WHAT I'M NOT UNDERSTANDING IS THAT
21 IN THE MECHANISMS IN WHICH FULL APPLICATIONS ARE
22 SUBMITTED, WHAT'S THE DIFFERENCE IN WHAT THE
23 REVIEWERS ARE ASKED TO LOOK AT AND EVALUATE IN THIS
24 INITIAL VOLUME CONTROL MODE COMPARED TO WHAT HAPPENS
25 WITH A FULL REVIEW? THEY'RE SITTING WITH THE SAME

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APPLICATION, BUT THEY'RE TREATING THEM DIFFERENTLY OR APPROACHING THEM DIFFERENTLY OR LOOKING AT DIFFERENT ELEMENTS OF THEM? WHAT'S THE DIFFERENCE?

DR. SAMBRANO: WE'LL GO OVER THAT.

DR. YAMAMOTO: OKAY.

DR. SAMBRANO: I HAVE THE SLIDES FOR EACH PROGRAM IN TERMS OF THE CRITERIA THAT WE'LL UTILIZE.

OTHER QUESTIONS? GOOD. OKAY. NEXT SLIDE.

ALL RIGHT. SO THIS IS AN EXAMPLE. SO AS I MENTIONED, THE METHODOLOGY THAT WE ARE PROPOSING IS ONE THAT UTILIZES TWO DIFFERENT MEASURES. ONE IS SELECTION AND THE OTHER IS RANKING. SO THE SELECTION IS MEASURING THE BREADTH OF INTEREST ACROSS REVIEWERS, AND THE RANK HELPS MEASURE THE INTENSITY OF THAT INTEREST. HOW WELL DO THEY LIKE IT IF THEY DO?

AND SO WE'LL GO THROUGH AN EXAMPLE HERE OF THEN HOW WE BRING THOSE TOGETHER TO CREATE A COMPOSITE SCORE FOR EACH OF THESE. AND SO WE'RE GOING TO USE PDEV IN TERMS OF THE SELECTION AND SCORING.

SO IN THIS CASE WE HAVE EACH APPLICATION IS ASSIGNED TO FOUR DIFFERENT REVIEWERS THAT IS PRESENT IN THE BATCH OF FOUR DIFFERENT REVIEWERS.

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1 EACH REVIEWER IS ASSIGNED 15 APPLICATIONS AND
2 SELECTS AND RANKS THEIR TOP 5. SO THEN THOSE
3 SELECTIONS AND RANKS ARE CONVERTED INTO A COMPOSITE
4 SCORE. AND SO IN THIS TABLE YOU SEE THE FOUR
5 DIFFERENT REVIEWERS NOTED AS GWG REVIEWER 1, 2, 3,
6 AND 4. IN TERMS OF SELECTIONS, REVIEWERS 1, 2, AND
7 4 SELECTED THIS EXAMPLE APPLICATION. AND SO,
8 THEREFORE, IT RECEIVES A POINT FOR EACH OF THOSE
9 SELECTIONS. AND SO WE THEN CALCULATE THE POINTS PER
10 MAXIMUM, MEANING THIS APPLICATION RECEIVED THREE
11 SELECTIONS OUT OF FOUR POSSIBLE, AND THEN IT'S
12 WEIGHTED 60 PERCENT. SO 60 PERCENT TIMES THREE OUT
13 OF FOUR. AND I'LL TELL YOU WHY THE WEIGHTING IN
14 JUST A SECOND.
15 THE RANKING, WE TAKE THE RANK. SO
16 REVIEWER 1 IN THIS EXAMPLE RANKED THIS APPLICATION
17 AS THEIR TOP ONE. THIS IS THEIR NO. 1. SO IT GETS
18 FIVE POINTS. THE SECOND REVIEWER RANKED THIS AS
19 THEIR SECOND FAVORITE. AND SO IT GETS FOUR POINTS.
20 THE THIRD REVIEWER DID NOT SELECT IT, SO, THEREFORE,
21 IT DOESN'T GET RANKED. AND THE FOURTH REVIEWER ALSO
22 RANKED IT AS THEIR TOP APPLICATION. AND SO THE
23 RANKINGS OF 1, 2, 3, 4, 5 ARE CONVERTED TO POINTS AS
24 NOTED WITH THE TOP ONE BEING 5 DOWN TO THE FIFTH
25 RANK BEING A 1. SO IT KIND OF IS INVERSE. AND SO

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1 THE APPLICATION RECEIVES 14 OUT OF 20 POSSIBLE
2 POINTS.
3 AND THEN THAT IS MULTIPLIED BY A
4 40-PERCENT WEIGHTING. AND SO THE WEIGHTING OF 60 TO
5 40 PERCENT PRIORITIZES THE SELECTION FOR BREADTH OF
6 INTEREST BY 60 PERCENT OVER THE VERY SPECIFIC
7 RANKING WHICH WE WEIGHT AT 40 PERCENT. SO WORKING
8 OUT THE MATH ON THAT GIVES YOU AN EXAMPLE COMPOSITE
9 SCORE OF .73 FOR THIS APPLICATION. THE BEST
10 POSSIBLE SCORE, IF THIS WAS SELECTED BY EVERYONE AND
11 THEY ALL RANKED IT NO. 1, WOULD BE A COMPOSITE SCORE
12 OF 1. SO THAT WOULD BE THE BEST POSSIBLE SCORE.
13 THE COMPOSITE ALLOWS US TO DO THIS
14 WEIGHTING SO THAT WE CAN PRIORITIZE THE SELECTION
15 WHILE STILL BENEFITING FROM THE INFORMATION PROVIDED
16 IN THE RANK AND DOESN'T ALLOW THE RANKING TO
17 OUTWEIGH OTHER ELEMENTS. SO THIS IS WHAT THE
18 MODELING IN TERMS OF THE SUGGESTED BALANCE PROVIDES
19 FOR.
20 ALL RIGHT. NEXT SLIDE PLEASE. OKAY. SO
21 NOW LOOKING BACK AT THE OVERALL PROCESS --
22 DR. LEVITT: SORRY, GIL. IF YOU GO BACK
23 ONE SLIDE, I JUST HAVE A QUESTION. YOU HAVE THREE
24 OUT OF FOUR SAY MOVE FORWARD AND THEN THEY RANK THEM
25 AND YOU HAVE TWO 5S AND A 4. SO QUITE HIGH

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RANKINGS.

DR. SAMBRANO: RIGHT.

DR. LEVITT: DOES THE ONE REVIEWER WHO SAY NO AND THEREFORE ZERO HAVE -- WHAT'S THE RIGHT WORD? -- A HIGHER IMPACT ON WHETHER THIS GRANT IS GOING TO MOVE FORWARD OR NOT? BECAUSE IF YOU HAVE THREE OUT OF FOUR, THREE OUT OF FOUR RANK AT EITHER FIRST OR SECOND IN THEIR PILE, YOU HAVE ONE THAT SAYS NO, AND NOW YOUR COMPOSITE SCORE IS .73.

DR. SAMBRANO: RIGHT.

DR. LEVITT: I DON'T KNOW IF YOU FIGURED OUT WHETHER A .73 WOULD ALLOW THIS GRANT TO GET PUT FORTH OR NOT. I'M JUST WONDERING WHETHER ONE DISSENTER WOULD HAVE AN OVERLY NEGATIVE IMPACT ON THE GRANT MOVING FORWARD OR NOT.

DR. SAMBRANO: RIGHT. SO GIVEN THE OVERLAP AND THE VARIABILITY IN THE BATCHES, IT IS UNLIKELY FROM AT LEAST THE DATA THAT WE HAVE BECAUSE IT'S UNUSUAL FOR AN APPLICATION TO HAVE A VERY LARGE NUMBER OF SELECTIONS WHERE EVERYONE SELECTS IT. SO SOMETHING LIKE THIS I WOULD VIEW AS ONE THAT IS LIKELY TO ADVANCE AND DO VERY WELL. AND IT'S EVEN POSSIBLE THAT ONE THAT RECEIVES A SINGLE SELECTION AND RANKS VERY WELL IN THAT REVIEWER HAS THE POSSIBILITY TO ADVANCE.

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1 DR. LEVITT: OKAY. JUST SOMETHING TO KEEP
2 IN MIND IN TERMS OF WHETHER THE REALITY IS THAT ONE
3 DISSENTER CAN HAVE A MORE WEIGHTED IMPACT ON WHETHER
4 A GRANT MOVES FORWARD OR NOT. AS YOU SAY, IT'S HARD
5 TO KNOW UNTIL YOU KNOW WHAT THE MIX IS GOING TO BE,
6 RIGHT, IN TERMS OF THE -- OKAY. THANKS.
7 DR. SAMBRANO: UH-HUH.
8 DR. YAMAMOTO: IF I COULD JUST JUMP IN
9 HERE, GIL. SO YOU JUST SAID THAT IT'S POSSIBLE THAT
10 ONE APPLICATION THAT IS SELECTED BUT RANKED VERY
11 HIGH COULD STILL HAVE THE POSSIBILITY OF ADVANCING.
12 SO THAT WOULD BE ONE POINT ON THE SELECTION SIDE AND
13 FIVE POINTS ON THE RANK SIDE. IT SEEMS THAT THAT
14 WOULD END UP WITH A COMPOSITE SCORE THAT'S PRETTY
15 LOW.
16 DR. SAMBRANO: RIGHT.
17 DR. YAMAMOTO: I'M NOT SURE -- IT SEEMS
18 LIKE THE COMPOSITE SCORE LOSES A LOT OF INFORMATION
19 BECAUSE YOU JUST SAID THAT ONLY ONE -- AND HERE WE
20 HAVE A SITUATION WHERE ONLY ONE REVIEWER EVEN PICKS
21 IT OUT. THAT ONE REVIEWER HAPPENS TO LIKE IT A LOT,
22 BUT THE OTHERS DIDN'T EVEN SELECT IT. BUT YOU JUST
23 TOLD US THAT THAT WOULD STILL HAVE A CHANCE TO BE
24 SUPPORTED. YOU COULD DO THE SAME KIND OF MATH TO
25 SAY THAT A LARGE NUMBER OF REVIEWERS, LET'S SAY

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1 THREE, SELECTED AND THEY ALL GIVE IT SORT OF A
2 MEDIOCRE RANK. SO I'M JUST CONCERNED THAT BY MAKING
3 THIS COMPOSITE SCORE, YOU'RE LOSING THAT KIND OF
4 ENTHUSIASM LEVEL OF EVALUATION. AND HOW MUCH DOES
5 THE COMPOSITE SCORE REALLY MEAN? AND IF THAT'S THE
6 CRITERION BY WHICH THINGS MOVE FORWARD OR NOT, IS
7 THIS THE RIGHT WAY TO BE DOING THIS CALCULATION?
8 DR. SAMBRANO: RIGHT. SO THE COMPOSITE
9 SCORE THAT YOU GET IS GOING TO BE A RELATIVE NUMBER
10 BASED ON THE SELECTIONS MADE BY THE REVIEWERS. SO
11 ULTIMATELY WE DON'T KNOW WHERE THE CUTOFF IS GOING
12 TO BE. BUT THE MODELING OF IT SUGGESTS THAT CUTOFFS
13 CAN BE VERY CLEAR USING THIS METHOD, MEANING YOU CAN
14 DISTINGUISH THOSE APPLICATIONS THAT ARE GOING TO BE
15 FAVORED BY REVIEWERS VERSUS THOSE THAT ARE NOT.
16 WHERE IT ENDS UP BEING, WHETHER IT
17 REQUIRES AT LEAST TWO SELECTIONS, ONE SELECTION TO
18 ADVANCE A PARTICULAR SET OF APPLICATIONS, WE NEED TO
19 MAYBE MODEL THAT ITSELF. AND THAT MAY ACTUALLY BE
20 IN DATA WE HAVE WHERE I CAN MAYBE GIVE YOU A BETTER
21 ANSWER. I JUST DON'T HAVE IT. BUT IT ALSO WILL
22 DEPEND ON WHAT WE ULTIMATELY GET.
23 NOW, THE WEIGHTING IS IMPORTANT HERE AS
24 WELL BECAUSE, AS YOU SAID, THE LEVEL OF ENTHUSIASM
25 IS IMPORTANT, BUT AN APPLICATION THAT'S SELECTED

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1 MULTIPLE TIMES BY MORE REVIEWERS SUGGESTS THAT IT
2 REPRESENTS SOMETHING THAT INTERESTS MULTIPLE
3 REVIEWERS. BUT THE RANK BY A GIVEN REVIEWER, IF
4 THEY RANK IT HIGH, IT IS STILL REFLECTING THAT
5 ENTHUSIASM. IF THEY ARE MORE MEDIOCRE IN TERMS OF
6 THE RANK, THEN THE LIKELIHOOD OF ADVANCING MAY BE
7 LOWER. YEAH. I THINK THERE'S A LOT OF SCENARIOS
8 THAT WE CAN PLAY AROUND WITH AND UNDERSTAND MORE.
9 BUT IN TERMS OF HOW WE'VE COMPARED IT TO
10 OTHER METHODOLOGIES, THIS CONTINUES TO BE THE MORE
11 ROBUST IN DOING IMPORTANT THINGS. ONE, GIVING US A
12 CLEAR CUTOFF, PREDICTING WHICH APPLICATIONS THAT
13 DEMONSTRATE GOOD QUALITY, AGAIN, USING REAL DATA
14 FROM WHAT WE'VE DONE IN THE PAST, THOSE THAT ARE
15 LIKELY TO SCORE 85 OR ABOVE, MEANING THEY WOULD BE
16 RECOMMENDED BY THE GRANTS WORKING GROUP, AND
17 REMOVING THOSE APPLICATIONS THAT ARE NOT LIKELY TO
18 BE COMPETITIVE. AND SO WITH A LARGE NUMBER OF
19 PROPOSALS, THIS IS AT LEAST A CONFIDENCE OF 77 TO 80
20 PERCENT UP TO 99 AND 100 PERCENT CONFIDENCE USING
21 THIS METHODOLOGY.
22 DR. YAMAMOTO: I HAVE TO THINK ABOUT THIS
23 A LITTLE MORE, BUT OKAY. THANKS.
24 DR. SAMBRANO: UH-HUH. ALL RIGHT. SO
25 THIS IS REVIEWING KIND OF THE DIFFERENT STEPS ALONG

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1 THE SELECTION PROCESS. SO, AGAIN, HERE ILLUSTRATING
2 IT THROUGH EXAMPLE, IF YOU HAVE A POOL OF 200
3 APPLICATIONS, WE TAKE THAT POOL AND MAKE ASSIGNMENTS
4 TO A SET NUMBER OF REVIEWERS. AND SO LET'S SAY 20
5 REVIEWERS THAT WILL THEN EACH GET A BATCH OF 30
6 APPLICATIONS. THE GRANTS WORKING GROUP PROCEEDS
7 WITH MAKING SELECTIONS AND RANKING THOSE SELECTIONS
8 SO THAT WE CAN DERIVE COMPOSITE SCORES.
9 THE COMPOSITE SCORES SHOULD ALLOW US TO
10 SEE A NATURAL BREAK BETWEEN THOSE THAT SCORE WELL
11 AND THOSE THAT DON'T AND THAT ALIGN AROUND OUR
12 TARGET. IF WE DO NOT SEE THAT, THERE ARE A COUPLE
13 OF WAYS IN WHICH WE CAN TIE-BREAK IF IT'S NECESSARY.
14 SO WE CAN USE THE HIGHER NOMINATION COUNT WAS ONE
15 STEP. IF THAT DOESN'T WORK, THOSE THAT RECEIVED THE
16 HIGHER RANKINGS OR A TOP RANKING CAN BE USED TO
17 BREAK A TIE. AND IF THAT DOESN'T WORK, THEN WE, AS
18 INDICATED IN THIS DIAGRAM, RESORT TO A SECOND ROUND
19 OF SELECTION WITH THAT GROUP THAT CANNOT BE
20 DISCERNED UNTIL THEN WE GET A CLEAR SEPARATION.
21 SO FOR THOSE THAT ADVANCE, SO THE TOP 40
22 TO 50 WITH CLEAR SEPARATION WOULD GO TO THEN THE
23 FULL MERIT REVIEW. SO THAT MEANS THAT WE WOULD MAKE
24 FULL ASSIGNMENTS, EXPECT A CRITIQUE, AND THEY WOULD
25 BE DISCUSSED BY THE PANEL AS WE TYPICALLY DO WITH

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1 THE GRANTS WORKING GROUP.
2 THOSE THAT DON'T MAKE IT, THE CIRM TEAM
3 AND PRESIDENT CAN REVIEW THOSE, AND THE PRESIDENT
4 CAN MAKE SELECTIONS TO ADVANCE A LIMITED NUMBER UP
5 TO THREE. AND THE BASIS FOR THAT SELECTION WOULD
6 STILL BE ALIGNED WITH THE GUIDING PRINCIPLES THAT I
7 WILL SHOW YOU IN SUBSEQUENT SLIDES. FOR ANYTHING
8 THAT THE PRESIDENT BELIEVES MIGHT HAVE BEEN MISSED
9 OR THAT WE SEE AS HAVING A GOOD ALIGNMENT AND
10 PROMISING PROJECT ALONG THOSE PRINCIPLES. AND SO
11 THE PRESIDENT HAS THAT OPTION.
12 NEXT SLIDE PLEASE. OKAY. SO WE'RE GOING
13 TO GO THROUGH THESE ONE AT A TIME STARTING WITH
14 CLIN2. YOU HAVE SEEN THE GUIDING PRINCIPLES. SO
15 THESE ARE JUST REITERATING WHAT SHYAM SHOWED YOU IN
16 TERMS OF THE TRANSFORMATIVE IMPACT FOR PATIENTS,
17 ADDRESSING BARRIERS FOR ACCESS AND AFFORDABILITY,
18 AND SO ON. AND THEN BELOW THAT YOU SEE THE CRITERIA
19 FOR THE GRANTS WORKING GROUP SELECTION, WHICH WE
20 COLLECTIVELY TERM THE SCIENTIFIC VALUE.
21 SO WHAT THESE ELEMENTS REPRESENT, THESE
22 FIRST THREE BULLETS, IMPACT OF THE PROPOSED
23 TREATMENT FOR PATIENTS IF SUCCESSFULLY DEVELOPED,
24 SIGNIFICANCE OF THE UNMET NEED, PRACTICALITY AND
25 FEASIBILITY OF THE PROPOSED TREATMENT TO BE ADOPTED

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1 BY PATIENTS AND HEALTHCARE PROVIDERS ARE THE EXACT
2 SAME AS IN THE FULL REVIEW CRITERIA THAT THE GRANTS
3 WORKING GROUP USES FOR THEIR SCIENTIFIC EVALUATION.
4 BUT HERE WE'RE FOCUSING THE REVIEWERS ON THE OVERALL
5 VALUE. IS THIS SOMETHING THAT IS WORTH FURTHER
6 CONSIDERATION BECAUSE IT HAS THE PROMISE OF ACTUALLY
7 HAVING A TRANSFORMATIVE IMPACT?
8 AND THE OTHER IMPORTANT ELEMENT,
9 PARTICULARLY FOR THE CLIN APPLICATIONS, IS THE STAGE
10 READINESS OF THIS PROJECT TO ADVANCE AND
11 SUCCESSFULLY COMPLETE A CLINICAL TRIAL? SO THAT
12 WOULD BE THE FOCUS IN THE ASK OF THE GRANTS WORKING
13 GROUP REVIEWERS AT THIS VOLUME CONTROL STAGE OF
14 REVIEW.
15 THEY WOULD EXPAND TO THE OTHER ELEMENTS,
16 AND I'LL SHOW YOU THAT IN A SUBSEQUENT SLIDE, OF THE
17 FULL CRITERIA FOR THE FULL REVIEW IF THEY DO
18 ADVANCE. NEXT SLIDE PLEASE.
19 ALL RIGHT. SO THIS IS THE SAME THING
20 SHOWING YOU THE GUIDING PRINCIPLES NOW FOR THE PDEV
21 PROGRAM THAT WERE PRESENTED BY SHYAM. AND THEN HOW
22 THEY INFORM, THEN, THE SCIENTIFIC VALUE. AND ONCE
23 AGAIN, THESE BULLET POINTS THAT SHOW THE POTENTIAL
24 TO PROVIDE A MEANINGFUL AND SUBSTANTIAL IMPROVEMENT
25 IN CLINICAL OUTCOMES, IMPACT OF ADDRESSING UNMET

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1 MEDICAL NEED, POTENTIAL FOR MORE ACCESSIBLE,
2 AFFORDABLE COMPARED TO AVAILABLE TREATMENTS, AND
3 FEASIBILITY AND PRACTICALITY OF THE THERAPY'S UPTAKE
4 BY PATIENTS, CAREGIVERS, AND HEALTHCARE SYSTEM ARE
5 THE EXACT SAME BULLETS THAT EXIST UNDER THE FIRST
6 VALUE CRITERION THAT THE REVIEWERS HAVE. AND IN THE
7 SAME WAY, AGAIN, FOCUSED ON THAT ELEMENT FOR THIS
8 STAGE OF THE REVIEW.
9 CHAIRMAN FISCHER-COLBRIE: PAT.
10 DR. LEVITT: SO, GIL, THIS IS WHERE MY
11 DISCONNECT IS AGAIN. AND I DON'T WANT TO
12 REPROSECUTE THE ISSUE AROUND AFFORDABILITY. BUT THE
13 THERAPY'S POTENTIAL TO BE MORE ACCESSIBLE AND
14 AFFORDABLE COMPARED TO AVAILABLE TREATMENTS OR
15 THERAPEUTICS CURRENTLY IN CLINICAL DEVELOPMENT IS
16 NOT WHAT MY INTERPRETATION OF WHAT THE DISCUSSION
17 WAS IN WHICH THERE WOULD BE A PLAN FOR HOW THE
18 INVESTIGATORS WOULD ENGAGE IN UNDERSTANDING WHAT THE
19 CHALLENGES ARE IN TERMS OF AFFORDABILITY, ET CETERA.
20 THIS IS -- THIS TO ME, WHEN I READ THIS AS AN
21 INVESTIGATOR, SAYS I NEED TO DEMONSTRATE IN MY
22 APPLICATION HOW WHAT I'M PROPOSING IS GOING TO BE
23 MORE AFFORDABLE THAN WHAT THE CURRENT TREATMENTS ARE
24 FOR A PARTICULAR DISEASE OR DISORDER. NOT A PLAN OF
25 HOW I'M GOING TO ENGAGE ONCE I GET THE APPLICATION,

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1 BUT RATHER REAL PROPOSAL ABOUT HOW THIS IS GOING TO
2 BE CHEAPER, LESS EXPENSIVE.
3 AND SO THIS IS -- WE CAN GO ROUND AND
4 ROUND ON THIS, BUT THE CONVERSATION WE HAD AND THIS
5 AS A CRITERION ARE NOT THE SAME.
6 DR. SAMBRANO: YEAH. YES. SO WE HAVE NOT
7 INCORPORATED THE DISCUSSION INTO THIS. THIS
8 REPRESENTS WHAT'S IN THE CURRENT REVIEW CRITERIA AND
9 PROGRAM ANNOUNCEMENT, BUT I THINK THE DISCUSSION
10 WE'VE HAD, I THINK, INFORMS WHAT WE WOULD PUT IN THE
11 NEW PROGRAM ANNOUNCEMENT, I THINK, INCORPORATING THE
12 COMMENTS THAT YOU PROVIDED. SO DEFINITELY RECOGNIZE
13 THAT.
14 DR. PATEL: AND, PAT, TO THAT POINT,
15 THAT'S A WONDERFUL POINT THAT YOU'RE RAISING AND
16 CLEARLY APPROPRIATE. AND THIS IS WHERE IN THE PDEV
17 APPLICATIONS AND THE REVIEWS PARTICULARLY THE
18 AFFORDABILITY ELEMENT IN THIS INSTANCE REALLY SORT
19 OF FOCUSES ON MANUFACTURING, THE ROUTE OF
20 ADMINISTRATION, AND THOSE SORTS OF THINGS RATHER
21 THAN SORT OF THE AFFORDABILITY THAT YOU'RE HINTING
22 AT, WHICH IS THIS ASPECT OF IS A PAYER GOING TO PAY
23 FOR IT KIND OF THING.
24 DR. LEVITT: OKAY. WELL, I MEAN MY
25 CONCLUSION THAT THIS -- THE CONVERSATIONS WERE LONG

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AND COMPLICATED. THOSE OF US WHO ARE ON THE BOARD ARE INTERPRETING IT IN OVERLAPPING BUT NOT IDENTICAL WAYS. AND THE ONE THING WE WANT TO TAKE OUT OF THIS TO BRING TO THE FULL BOARD IS SOME CLARITY AND TRANSPARENCY ABOUT WHAT THE TEAM MEANS BY THIS AND WHAT THE INVESTIGATORS ARE EXPECTED TO ACHIEVE IN THEIR APPLICATION. THAT'S MY FEELING. WHATEVER THAT IS, IT'S A LONGER CONVERSATION.

DR. SAMBRANO: YEAH.

MS. MANDAC: MONICA HAS HER HAND RAISED.

DR. CARSON: I JUST WANT TO DOUBLE-DOWN ON PAT'S COMMENT BECAUSE IT JUST RECAPTURES THE THINGS I WAS TRYING TO BRING UP. ACKNOWLEDGING ALL THE WISDOM OF THE GWG, THAT'S NOT THE ISSUE EXPLICITLY. IT'S THAT, AS SOMEBODY WHO SUBMITS GRANTS ALL THE TIME, YOU POUR OVER THIS, YOU TRY TO DO THIS. AND THEN JUST LISTENING TO HOW ALL WE HEARD IT, AS THE GWG EVOLVES, OVER TIME MEMBERS CHANGE, ALL OF THIS KIND OF STUFF, THESE ARE THE THINGS THAT GET LOST. AND WE ALL LIKE THE PRINCIPLES. WE ALL ARE AGREEING ON THAT, BUT I KEEP SAYING THE DEVIL IS IN THE DETAILS. AND THIS IS NOT JUST SOMETHING SIMPLY TO SAY WE'RE GOING TO WORDSMITH IT OUT. THIS REALLY NEEDS TO BE FOCUS GROUPED AND REALLY WORKED OUT IN DETAILS BECAUSE IT IS PUBLIC FACING, IT IS SOMETHING

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1 WE HAVE TO BE SENSITIVE TO, IT'S CLEARLY QUITE
2 READILY MISUNDERSTOOD BY VERY INTELLIGENT PEOPLE
3 COMING FROM ALL THESE DIFFERENT THINGS AND YET WE
4 ALL AGREE ON THE ASPIRATIONAL GOALS. SO JUST WANTED
5 TO DOUBLE-DOWN ON THAT COMMENT.
6 DR. SAMBRANO: THANK YOU. APPRECIATE
7 THAT. WE CAN GO TO THE NEXT SLIDE.
8 ALL RIGHT. SO THEN THIS IS THE SAME THING
9 FOR DISC4, NOTING, AGAIN, THE GUIDING PRINCIPLES
10 ABOVE AND THEN HOW THEY INFORM THE OVERALL
11 SCIENTIFIC VALUE. IN THIS CASE FOR DISC4, WE'RE
12 LOOKING AT THE IMPACT OF THE PROPOSED PROJECT IN
13 ADVANCING UNDERSTANDING OF HUMAN DISEASE, GENERATING
14 DATA RESOURCES, OR WHERE OUTCOMES RAPIDLY ADVANCE
15 THE THERAPEUTIC OR A BIOMARKER. BUT WE'RE ALSO
16 INTRODUCING THE ELEMENTS OF INNOVATION RELATIVE TO
17 THE CURRENT STATE OF RESEARCH, SUCH AS APPLYING
18 NOVEL FRAMEWORKS TO STUDY DISEASE, CUTTING ACROSS
19 SILOS, AND APPLYING THE UNIQUE SYNERGY OF
20 TECHNOLOGIES, OR UTILIZING INNOVATIVE APPROACHES.
21 AND, OF COURSE, THE RESPONSIVENESS OF THE
22 APPLICATION TO THE FOCUS AREA THAT WE DECIDE ON
23 WOULD ALSO BE AN ELEMENT THAT WOULD BE CONSIDERED IN
24 THE OVERALL SCIENTIFIC VALUE OF THE PROPOSALS AT
25 THIS STAGE.

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1 OKAY. NEXT SLIDE. OKAY. AND SO FOR
2 THOSE APPLICATIONS THAT ADVANCE, SO NOW THE NARROW
3 POOL, THEY ARE EVALUATED AND SCORED BY THE FULL
4 PANEL, AS MENTIONED. AND THE EVALUATIONS AND SCORES
5 ARE THEN NOW BASED ON THE FULL SET OF REVIEW
6 CRITERIA. AND THOSE SPECIFIC REVIEW CRITERIA ARE
7 DETAILED IN THE MEMO. THEY'RE A BIT MORE EXTENSIVE
8 THAN FOR EACH PROGRAM ON THIS SLIDE, AND THEY ALSO
9 VARY A BIT FOR EACH PROGRAM BECAUSE THEY'RE TAILORED
10 TO EACH OF THE DIFFERENT FUNDING OPPORTUNITIES. BUT
11 IN GENERAL THEY COVER THESE ELEMENTS: THE OVERALL
12 VALUE PROPOSITION AND, OF COURSE, SIGNIFICANCE AND
13 IMPACT, WHICH IS ESSENTIALLY WHAT THE FOCUS OF THE
14 VOLUME CONTROL ELEMENT IS, BUT THEY CONTINUE TO
15 CONSIDER THAT AS THEY MOVE FORWARD INTO THE FULL
16 REVIEW. INNOVATION APPLIES TO THE DISC4 ONLY. AND
17 THEN THE OTHERS ARE RATIONALE OF THEIR PROPOSAL, THE
18 PROJECT PLAN, AND HOW THEY'VE DESIGNED IT, THE
19 PROJECT TEAM, AND THE RESOURCES AVAILABLE TO THE
20 PROJECT, AND POPULATION IMPACT. POPULATION IMPACT
21 REFERS TO THE IMPACT OF ADDRESSING THIS DISEASE ON
22 THE POPULATION THAT IS AFFECTED. SO THAT FOR A
23 CLINICAL TRIAL APPLICATION, FOR EXAMPLE, CAN INCLUDE
24 UNDERSTANDING THE AFFECTED POPULATION THAT EXISTS.
25 THIS IS PARTICULARLY IMPORTANT FOR CERTAIN RARE

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1 DISEASES, UNDERSTANDING THE TYPES OF PATIENTS,
2 NUMBER OF PATIENTS THAT ARE AFFECTED, ALSO WHAT THEY
3 HAVE DONE AS APPLICANTS TO UNDERSTAND THAT
4 POPULATION AND HOW THEY WILL ENROLL, DO OUTREACH
5 ACTIVITIES TO BRING THAT BROADER POPULATION INTO A
6 TRIAL.
7 AT THE DISCOVERY STAGE, IT IS SIMPLY
8 UNDERSTANDING HOW DIFFERENCES THAT MIGHT IMPACT
9 OUTCOMES, SUCH AS SEX DIFFERENCES OR OTHER ELEMENTS
10 IN THE POPULATION IN TERMS OF HOW A SPECIFIC DISEASE
11 OR CONDITION IS EXPRESSED IN THAT POPULATION ARE
12 CONSIDERED BY THE APPLICANTS.
13 NEXT SLIDE. ALL RIGHT. AND SO THEN THIS
14 IS THE LAST SLIDE IN THIS SECTION, AND IT'S JUST
15 FOCUSED ON ADDRESSING SOMETHING THAT WAS BROUGHT UP
16 BEFORE, WHY WE WOULD REQUEST A FULL APPLICATION IN
17 SOME INSTANCES. ONE OF THE MOST IMPORTANT IS THAT A
18 SURVEY FROM GRANTS WORKING GROUP REVIEWERS WHO HAVE
19 BEEN DOING POSITIVE SELECTION AND SIMILAR APPROACHES
20 INDICATES THAT THE VAST MAJORITY, 95 PERCENT, USE
21 THE FULL APPLICATION IN DOING THEIR ASSESSMENTS TO
22 MAKE FINAL SELECTIONS. IT ENSURES THAT ELIGIBILITY
23 CAN BE FULLY DETERMINED AND MAKES IT UNLIKELY THAT
24 AN INELIGIBLE APPLICATION TAKES UP A VALUABLE SLOT.
25 WE OFTEN RUN INTO ISSUES WITH ELIGIBILITY, AND WE

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1 DON'T WANT THAT ELEMENT TO BE A FACTOR IN
2 DETERMINING WHAT ULTIMATELY ADVANCES.
3 IT ENSURES THAT SELECTED PROJECTS ARE AT
4 THE APPROPRIATE STAGE OF READINESS, AND THIS IS
5 PARTICULARLY TRUE FOR PDEV AND CLIN2 IN HAVING THEIR
6 REGULATORY, CMC, AND OTHER PRODUCT DEVELOPMENT
7 ELEMENTS IN PLACE, AND ALSO THE TIME FROM SUBMISSION
8 TO AWARD IS SIGNIFICANTLY SHORTER AND MITIGATES
9 PROJECT CHANGES AND STALENESS AS MANY REVIEWERS
10 REFER TO IT OVER TIME.
11 SO I WILL END THIS PORTION HERE. AND IF
12 THERE ARE QUESTIONS BEFORE WE MOVE ON, PLEASE FEEL
13 FREE.
14 CHAIRMAN FISCHER-COLBRIE: ANY QUESTIONS
15 OR COMMENTS? OKAY. LET'S CONTINUE.
16 DR. PATEL: OKAY. SO WE'RE GOING TO GO TO
17 THE LAST STEP IN THE APPLICATION LIFE CYCLE TO WALK
18 THE BOARD THROUGH OUR PROPOSAL FOR HOW THE GUIDING
19 PRINCIPLES ARE INCORPORATED INTO JUST PROVIDING A
20 ROBUST INFORMATION PACKAGE FOR THE ARS IN MAKING
21 THEIR FUNDING DECISIONS. AND SO, AGAIN, JUST TO
22 ITERATE, WE'RE ON THAT GREEN PART NOW, THE FUNDING
23 DECISION FOR THE ARS AND PUTTING IT IN A RED BOX
24 MAKING IT MORE OBVIOUS.
25 SO AS YOU KNOW, HISTORICALLY THE CIRM TEAM

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1 HAS PROVIDED RECOMMENDATIONS TO ASSIST THE
2 APPLICATION REVIEW SUBCOMMITTEE IN MAKING
3 WELL-INFORMED FUNDING DECISIONS. BUT ULTIMATELY THE
4 ARS CONSIDERS MANY DIFFERENT THINGS IN HOW IT
5 CHOOSES TO FUND WHICH APPLICATIONS. SO THERE'S
6 GOING TO BE THE GRANTS WORKING GROUP REVIEW
7 SUMMARIES, FIRST AND FOREMOST. THERE MAY BE
8 PROGRAMMATIC FACTORS. WHAT'S THE PROGRAM PRIORITY,
9 BUDGET OF THE OVERALL PROGRAM FOR THAT YEAR, AND SO
10 ON, THE RECOMMENDATIONS FROM THE CIRM TEAM. THAT
11 MAY BE SPECIFIC FUNDING RECOMMENDATIONS FOR THOSE
12 APPLICATIONS IN THE POOL. AND LASTLY, THE RICH
13 PUBLIC COMMENTS THAT ARE UNIQUE TO OUR FUNDING
14 AGENCY.
15 AND SO IN THE SUBSEQUENT SLIDE, I'LL
16 DESCRIBE HOW THESE GUIDING PRINCIPLES ARE ASSESSED
17 AND LEVERAGED IN THE CIRM TEAM RECOMMENDATIONS. SO
18 WHAT WE'RE PROPOSING TO DO IS THAT FOR -- IN ORDER
19 TO GIVE A MORE ROBUST INFORMATION PACKAGE, FOR EVERY
20 APPLICATION THAT SCORES AN 80 OR HIGHER BY THE
21 GRANTS WORKING GROUP OR THAT HAS A MINORITY REPORT,
22 WE WILL GENERATE A GUIDING PRINCIPLE SUMMARY FOR THE
23 ARS. WHAT THIS IS GOING TO DO IS DISTILL SOME
24 POINTS OF THE APPLICATION OR THE GRANTS WORKING
25 GROUP OR THE ANA SPECIALIST REVIEWER SENTIMENT INTO

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1 THOSE GUIDING PRINCIPLE CATEGORIES.
2 SO, FOR EXAMPLE, WITH TRANSFORMATIVE
3 CLINICAL IMPACT, PROVIDING A SUCCINCT SUMMARY OF THE
4 OVERALL GWG SENTIMENT ON THIS TOPIC. AND THEN ON
5 THE CIRM TEAM SIDE, PROVIDING A COMPARISON TO OUR
6 ACTIVE PORTFOLIO AND THE EXTERNAL LANDSCAPE WITHIN
7 THAT SAME INDICATION.
8 ON THE ACCESS AND AFFORDABILITY POINT,
9 AGAIN TO PAT'S POINT, MAKING SURE THIS IS REALLY
10 CLARIFIED. THIS IS GOING TO BE A SUMMARY OF THE
11 GRANTS WORKING GROUP SENTIMENT ON THIS TOPIC FOR
12 THAT APPLICATION. THE SUMMARY OF THE ANA REVIEWER
13 SENTIMENT. SO FOR A CLIN2 APPLICATION, THERE IS A
14 SPECIALIST EXPERT ON ACCESS AND AFFORDABILITY
15 CONSIDERATIONS WHO IS PROVIDING A NARRATIVE, AND
16 WE'D INCORPORATE THE SUMMARY OF THEIR SENTIMENT.
17 AND THEN OUR KNOWLEDGE OF ANY ASPECTS OF THE
18 APPROACH THAT COULD BE RELEVANT.
19 AND THEN LASTLY, DISEASE REPRESENTATION.
20 SO HERE THE CIRM TEAM WILL BE PREPARING INFORMATION
21 ABOUT HOW THIS PARTICULAR PROJECT OR ITS INDICATION
22 WOULD ENRICH THE CIRM PORTFOLIO IN TERMS OF IS THAT
23 DISEASE INDICATION REPRESENTED. AND OVERALL WHAT'S
24 THE PROPORTION OF THAT OVERALL DISEASE AREA? SO,
25 FOR EXAMPLE, IF IT'S A SPECIFIC NEUROLOGICAL

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1 DISORDER THAT'S NOT REPRESENTED IN OUR PORTFOLIO, WE
2 WOULD INDICATE THAT. AND WE WILL ALSO INDICATE OUR
3 BROADER DISEASE AREA OF NEURO DISORDERS HAD X
4 PERCENTAGE OF THE OVERALL ACTIVE PORTFOLIO, WHICH
5 ALL MAY BE IMPORTANT INFORMATION THAT CAN BE USEFUL
6 FOR THE BOARD TO DECIDE ACROSS A BATCH OF
7 APPLICATIONS THAT IT'S SEEING HOW IT MAY DIRECT ITS
8 FUNDING DECISIONS.
9 ON TOP OF THAT, SO FOR DISC4, IT'S A
10 SIMILAR SORT OF SUMMARY, BUT TAILORED TO THE GUIDING
11 PRINCIPLES FOR THAT PROGRAM. AND THEN THAT WOULD BE
12 PART -- ONE PART OF THE OVERALL PACKAGE OF
13 INFORMATION PRESENTED TO THE ARS. SO IN ADDITION TO
14 THE GUIDING PRINCIPLES SUMMARY, IF THE CIRM TEAM HAS
15 SPECIFIC FUNDING RECOMMENDATIONS THAT ARE BASED ON
16 THINGS LIKE THE AVAILABLE PROGRAM BUDGET, THE GRANTS
17 WORKING GROUP SCORE AND COMMENTS, THE GUIDING
18 PRINCIPLES SUMMARY, THE PERFORMANCE OF THE PRIOR
19 AWARDEE, AS WELL AS NEW INFORMATION AVAILABLE TO
20 CIRM AFTER GRANTS WORKING GROUP REVIEW. ALL OF THAT
21 WILL BE SUMMARIZED IN A MEMO. SO THIS IS IF THE
22 CIRM TEAM HAS SPECIFIC FUNDING RECOMMENDATIONS FOR
23 THE BATCH OF APPLICATIONS BEING CONSIDERED BY THE
24 ARS, WE INCORPORATE ALL THIS INFORMATION INTO A
25 NARRATIVE THAT WILL BE PROVIDED TO THE BOARD AS A

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1 MEMO.
2 SO ULTIMATELY THE BOARD WOULD HAVE, TO
3 REITERATE, THE GUIDING PRINCIPLES SUMMARY, THE CIRM
4 TEAM RECOMMENDATION, THE GRANTS WORKING GROUP
5 SUMMARY, AS WELL AS ANY PUBLIC COMMENTS THAT HAVE
6 BEEN MADE FOR THAT BATCH OF APPLICATIONS ON HAND TO
7 MAKE FUNDING DECISIONS.
8 THIS IS AN EXAMPLE OF A MEMO THAT WILL BE
9 PROVIDED TO THE BOARD FOR A PARTICULAR APPLICATION
10 CYCLE FOR A PARTICULAR PROGRAM. SO IT WOULD PROVIDE
11 STRUCTURED INFORMATION INCLUDING WHAT'S THE
12 AVAILABLE PROGRAM BUDGET, HOW MUCH OF IT HAS BEEN
13 UTILIZED IN PREVIOUS CYCLES IN THIS FISCAL YEAR,
14 WHAT WAS THE SCORING BREAKDOWN FOR THE APPLICATIONS,
15 AND WHAT IS THE CIRM TEAM RECOMMENDATION, AS WELL AS
16 WHAT I NOTED PREVIOUSLY, FOR ANY GIVEN APPLICATION,
17 WE WOULD PROVIDE A CIRM TEAM RECOMMENDATION FOR
18 FUNDING, AS WELL AS A GUIDING PRINCIPLE SUMMARY. SO
19 ALL OF THIS WILL BE PACKAGED INTO A MEMO THAT WOULD
20 HELP THE BOARD MAKE ITS DECISIONS.
21 MR. TOCHER: PAT'S HAND IS RAISED.
22 DR. LEVITT: YEAH. I HAVE -- CAN YOU GO
23 BACK ONE SLIDE?
24 DR. PATEL: SURE.
25 DR. LEVITT: NO. BACK AGAIN. THERE. I

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1 DON'T KNOW WHAT'S -- TELL ME WHAT YOU MEAN BY BULLET
2 5, NEW INFORMATION AVAILABLE TO CIRM AFTER GWG
3 REVIEW.
4 DR. PATEL: THAT'S A GREAT QUESTION. SO
5 THAT MAY BE -- SO WHAT WE'RE DOING HERE IS WE'RE
6 COMPILING INFORMATION THAT WOULD BE RELEVANT TO THE
7 APPLICATION. SO IT'S THAT LAST BULLET POINT. THERE
8 MAY BE SOME DEVELOPMENT IN THE FIELD OR THERE MAY BE
9 SOME DEVELOPMENT, A NEW THERAPY HAS BEEN APPROVED.
10 I'LL GIVE YOU AN EXAMPLE OF THIS THAT MIGHT MAKE IT
11 A LITTLE BIT MORE TANGIBLE.
12 IN THE LAST PDEV ROUND THERE WAS AN
13 APPLICATION THAT GOT FUNDED WHERE A LOT OF ITS PLAN
14 WAS BASED ON A THERAPY THAT WAS IN PHASE 3 TRIALS
15 THAT MIGHT NOT BE INCORPORATED INTO ITS CLINICAL
16 PLAN. AND AFTER GWG REVIEW, BUT BEFORE ARS, THERE
17 WAS A READOUT ON THAT THERAPY THAT WOULD BE PART OF
18 THE TREATMENT PLAN FOR THIS PARTICULAR INDICATION.
19 AND WE WOULD PROVIDE A SUMMARY OF THAT INFORMATION
20 THAT COULD POTENTIALLY HELP THE BOARD. SO THAT MAY
21 BE ONE INSTANCE.
22 ANOTHER ONE MAY BE THAT THIS APPLICANT HAS
23 A CURRENT AWARD AND THAT THERE WAS SOME EVENT THAT
24 HAPPENED FOR AN AWARD THAT WASN'T CAPTURED WHEN THE
25 GRANTS WORKING GROUP WAS REVIEWING IT, AND WE

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1 PROVIDE THAT INFORMATION AS WELL.
2 DR. LEVITT: UH. OKAY. WELL, WE'RE
3 ALREADY OVER. I'M NOT GOING -- THERE'S SOME ISSUES
4 THERE, SHYAM, THAT WE SHOULD DISCUSS BECAUSE THE
5 QUESTION IS HOW THE INFORMATION IS GETTING TO THE
6 CIRM TEAM, IS IT VALIDATED, IS THE REVIEWERS ON THE
7 GWG INFORMED OF THIS NEW INFORMATION SO THEY CAN
8 WEIGH IN. IT SHOULD BE A RARE OCCURRENCE. AND AS
9 YOU KNOW, THERE ARE MANY FUNDING AGENCIES THAT DON'T
10 TAKE THIS INTO ACCOUNT BECAUSE THEY WANT A LEVEL
11 PLAYING FIELD, THAT IS. YEAH. I'LL STOP THERE, BUT
12 IT'S NOT CLEAR TO ME YET HOW THE KINDS OF WAYS IT
13 WOULD BE USED AND HOW ONE WOULD VERIFY THE
14 INFORMATION.
15 MR. TOCHER: MARIA HAS HER HAND RAISED.
16 CHAIRMAN FISCHER-COLBRIE: MARIA.
17 VICE CHAIR BONNEVILLE: HI. I JUST WANTED
18 TO ASK VITO. I THINK AT THE BOARD MEETING IT'S
19 CRITICAL FOR YOU TO THINK ABOUT AND TALK TO THE
20 BOARD ABOUT HOW THE EMPHASIS OF THE ARS MIGHT CHANGE
21 AND WHAT CHANGES PERHAPS IN PROCESSES AS WELL AS OUR
22 OWN METHOD FOR HOW WE TAKE APPLICATIONS UP IN THE
23 ORDERS, ET CETERA, BECAUSE THE RUNNING OF THE ARS IS
24 GOING TO BE DIFFERENT. AND I THINK IT'S CRITICAL
25 THAT THE BOARD AND THE ARS UNDERSTAND WHAT'S BEING

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ASKED OF THEM AT THIS POINT.

CHAIRMAN IMBASCIANI: MARK, MAY I RESPOND?

CHAIRMAN FISCHER-COLBRIE: PLEASE.

CHAIRMAN IMBASCIANI: YEAH. THANK YOU, MARIA. WE'VE ALREADY STARTED CONVERSATIONS WITH RELEVANT FOLKS TO GAME-PLAY THIS, IF YOU WILL. IT'S -- I THINK THE MOST OBVIOUS THING IS WITH RESPECT TO HOW ARS TAKES UP CLINICAL APPLICATIONS FOR FUNDING, WE'VE USUALLY CONSIDERED THEM, ACCEPTED THE RECOMMENDATION OF THE TEAM, AND THEN VOTED ON THEM ONE BY ONE. BUT I CAN SEE IMPLICATIONS FOR THAT GIVING THESE REPORT CARDS AND TEMPLATES WHERE THAT MAY NOT BE POSSIBLE BECAUSE THE ORDER OF THE APPLICATIONS MIGHT SUGGEST THAT WE WOULD EXHAUST OUR BUDGET BEFORE FULL CONSIDERATION WAS GIVEN TO ALL OF THEM AND THEREBY DEFEATING ONE OF THE PRINCIPLES OF THE REVISED REVIEW PROCESS.

SO THERE'S MORE TALK ABOUT THAT. I'M AWARE OF WHAT YOU SAID, AND WE STARTED WORKING ON IT ALREADY. THANK YOU.

VICE CHAIR BONNEVILLE: I THINK IT ALSO PLAYS INTO WHETHER OR NOT THERE ARE CAPS BY INDIVIDUAL ROUNDS, WHICH I KNOW IS SOMETHING THE TEAM IS LOOKING AT EXPLORING AND BRINGING TO THE BOARD IN JUNE. SO THAT WILL OBVIOUSLY ALSO HAVE AN

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IMPACT BECAUSE --

CHAIRMAN IMBASCIANI: GOOD POINT.

VICE CHAIR BONNEVILLE: -- IF IT'S THE FIRST REVIEW OF THE SEASON AFTER THE BUDGET, YOU COULD FUNDAMENTALLY FUND ALL OF THEM AND THEN YOU'VE EXHAUSTED YOUR BUDGET IN ONE FELL SWOOP. SO ALL OF THESE THINGS REALLY DO HAVE TO BE DISCUSSED, AND THERE ARE A FEW ARS MEMBERS ON THIS CALL. SO IT'S SOMETHING FOR US TO CONSIDER.

CHAIRMAN IMBASCIANI: GREAT. THANK YOU.

CHAIRMAN FISCHER-COLBRIE: HEY, I WANT TO DO A TIME CHECK HERE. WE'RE OBVIOUSLY 15 MINUTES OVER. I THINK JUST FROM A QUALITATIVE PERSPECTIVE, THE TEAM HAS DONE A HUGE AMOUNT OF WORK AND THERE'S TREMENDOUS PROGRESS. HAVING SAID THAT, I THINK THERE ARE DEFINITELY SOME CLARIFICATION POINTS THAT MERIT FURTHER DISCUSSION AND REVIEW. MY PERSONAL PREFERENCE WOULD BE TO HAVE A FOLLOW-ON MEETING TO HAVE THAT DISCUSSION PRIOR TO THE FULL CONSIDERATION FROM THE BOARD. BUT I WANTED TO DO A TIME CHECK HERE. I THINK THERE'S A FEW MORE SLIDES FOR REVIEW?

DR. PATEL: YEAH. THERE'S A COUPLE MORE SLIDES ON THE DISC4 PROGRAM AND ON THE PDEV PROGRAM UPDATES THAT WE CAN GO THROUGH IF THERE'S TIME.

CHAIRMAN FISCHER-COLBRIE: OKAY.

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1 MR. TOCHER: SO WE WILL LIKELY LOSE A
2 QUORUM IN A FEW MINUTES, PROBABLY 12:30 AT THE
3 LATEST. SO THAT WOULD LEAVE 14 MINUTES TO ADDRESS
4 THEM. BUT WE'RE COMING BACK REGARDLESS.
5 DR. THOMAS: YES.
6 DR. PATEL: SO WE CAN TEE THIS OFF AND
7 THEN HAVE A DISCUSSION ABOUT ALL THE POINTS THAT THE
8 SUBCOMMITTEE WOULD LIKE US TO ADDRESS. SO, PAT --
9 CHAIRMAN FISCHER-COLBRIE: CORRECT.
10 DR. PATEL: -- ON THIS ONE AS WELL.
11 SO THERE'S A COUPLE OF OTHER ITEMS THAT
12 RELATE TO DISC4 PROGRAM AND THE PDEV PROGRAM WHICH
13 WILL BE INCORPORATED INTO ANY CONCEPT PLAN
14 AMENDMENTS AND KEEP THOSE PROGRAMS ON TRACK FOR THE
15 NEXT FUNDING CYCLE SHOULD THE BOARD APPROVE THESE
16 CHANGES IN MARCH.
17 SO ON THE DISC4 SIDE, AS YOU PREVIOUSLY
18 NOTED AND TALKED A LITTLE BIT ABOUT, THERE'S A FOCUS
19 AREA AND THIS ROTATES. SO ONE FISCAL YEAR THE FOCUS
20 AREA IS SET BY THE NEURO TASK FORCE OF THE SCIENCE
21 SUBCOMMITTEE. AND IN THE NEXT FISCAL YEAR, THE CIRM
22 TEAM MAKES A RECOMMENDATION, AND THE BOARD SETS THE
23 FOCUS AREA.
24 AND SO FOR THE NEXT FISCAL YEAR CYCLE FOR
25 DISC4, THE CIRM TEAM IS MAKING A RECOMMENDATION PER

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1 THIS CADENCE FOR THE BOARD'S CONSIDERATION. AND
2 THAT IS TO MAKE THE FOCUS AREA IMMUNE TISSUE
3 INTERACTIONS IN DISEASE AND REPAIR. SO AS WE'RE ALL
4 AWARE, THE IMMUNE SYSTEM IS BEING MORE AND MORE
5 IMPLICATED IN PATHOLOGY, REPAIR, AND REGENERATIVE
6 MECHANISMS ACROSS MULTIPLE ORGAN SYSTEMS IN HUMAN
7 DISEASES. AND SO THIS IS A TIMELY TOPIC WHERE
8 MULTIDISCIPLINARY APPROACHES COULD REALLY ELUCIDATE
9 MORE UNDERSTANDING OF THAT IMMUNE AND ORGAN SYSTEM
10 INTERACTION AND COULD ALSO BENEFIT FROM A NETWORK OF
11 AWARDEES WHERE SHARING OF DATA AND METHODOLOGIES AND
12 RESOURCES COULD PROVIDE INSIGHTS ACROSS DISEASE AND
13 ORGAN SYSTEMS.
14 AND SO THAT'S THE NATURE -- THE RATIONALE
15 FOR PROPOSING THIS PARTICULAR TOPIC. AND I THINK
16 IT'S APPLICABLE TO A BROAD RANGE OF DISEASES
17 AFFECTING CALIFORNIANS. AND AS I NOTED, IT DOES
18 ALLOW FOR A MULTIDISCIPLINARY AND OMICS-DRIVEN
19 APPROACHES. SO I WILL STOP THERE FOR ANY COMMENTS
20 THAT WE CAN INCORPORATE INTO THE NEXT MEETING.
21 MR. TOCHER: MARIA.
22 VICE CHAIR BONNEVILLE: THE DISC -- I WANT
23 TO GO BACK TO ASKING MY COLLEAGUES. I THINK IN
24 THIS -- FOR THIS PROGRAM I'M NOT OPPOSED TO IT BEING
25 A FOCUSED AREA, NOT ALLOWING THINGS OUTSIDE OF WHAT

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1 YOU'VE ASKED FOR THE IMMUNE TISSUE INTERACTIONS AND
2 DISEASE AND REPAIR, WHICH I KNOW SO MUCH ABOUT, BY
3 THE WAY. BUT I'M NOT OPPOSED TO HAVING THAT BE THE
4 SOLE FOCUS AND NOT ALLOW OTHER INDICATIONS OR
5 MODALITIES TO COME IN. SO IF THAT'S SOMETHING THAT
6 MY COLLEAGUES THE BOARD ARE OKAY WITH, I THINK IT
7 STRENGTHENS THIS PROGRAM AND COULD BE PART OF THE
8 CONCEPT PLAN, BUT I THINK WE HAVE TO LET THE TEAM
9 KNOW THAT AND MAKE A RECOMMENDATION TO THE BOARD TO
10 HAVE THAT BROUGHT UP OR TALK ABOUT IT AT THE BOARD
11 MEETING.
12 CHAIRMAN FISCHER-COLBRIE: THANK YOU,
13 MARIA. ARE THERE OTHER SLIDES HERE OR WAS THAT THE
14 LAST SLIDE?
15 DR. PATEL: SO THIS IS FOR THE DISC4
16 SLIDE. IT'S ONLY A SLIDE FOR DISC4. SO IF THERE
17 ARE NO COMMENTS, WE CAN MOVE ON.
18 MR. TOCHER: YOU'RE GOOD.
19 CHAIRMAN FISCHER-COLBRIE: YEP.
20 DR. PATEL: THAT'S GREAT. AND SO THIS IS
21 THE LAST TOPIC, AND I APPRECIATE ALL THE FEEDBACK SO
22 FAR. THIS IS ON THE PDEV CYCLE RECURRENCE.
23 SO AS THE BOARD IS AWARE, THE PDEV PROGRAM
24 CURRENTLY SPECIFIES TWO APPLICATION CYCLES PER
25 FISCAL YEAR. AND IN OUR ANALYSIS OF THE FIRST TWO

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1 CYCLES, THE EXPECTED BENEFITS OF THOSE TWO CYCLES
2 PER YEAR ARE NOT BEING EITHER REALIZED OR NOT
3 ENTIRELY RELEVANT TO THIS PROGRAM.
4 SO SOME OF THE BENEFITS OF HAVING MULTIPLE
5 CYCLES PER FISCAL YEAR INCLUDE THAT THE PROGRAM IS
6 AVAILABLE WHEN THE PROJECT IS READY. HOWEVER, FOR
7 THE PDEV PROGRAM, WE ALLOW ENTRY AT ANY POINT ACROSS
8 THE PRECLINICAL DEVELOPMENT LIFE CYCLE. SO THIS MAY
9 NOT BE AS RELEVANT AS IN SOME OTHER PROGRAMS LIKE
10 CLIN2 WHERE IND CLEARANCE AND THEN BEING ABLE TO
11 APPLY MIGHT MAKE SENSE.
12 AN OPERATIONAL ELEMENT IS THAT THE
13 APPLICATION, YOU WOULD EXPECT THAT IF YOU HAD
14 MULTIPLE CYCLES, USE PERMISSION WOULD RESULT IN A
15 REDUCED DELAY IF YOU HAD MULTIPLE CYCLES.
16 FOR THE PDEV CYCLE, GIVEN THE NUMBER OF
17 REVIEWS THAT WE DO PER YEAR ACROSS CIRM AND THE
18 LENGTH OF THE CYCLE, THE CYCLES ACTUALLY OVERLAP FOR
19 PDEV. AND SO IT DOESN'T REALLY ALLOW SOMEONE TO
20 RESUBMIT IMMEDIATELY FOR THE NEXT CYCLE.
21 AND LASTLY, IF YOU HAVE MULTIPLE CYCLES,
22 YOU WOULD BE ABLE TO BATCH THE APPLICATIONS AND
23 AWARDS OVER THE FISCAL YEAR. HOWEVER, AS MARIA
24 NOTED BEFORE, BECAUSE WE DON'T HAVE CYCLE CAPS,
25 THERE'S A HIGH CHANCE THE FIRST FISCAL YEAR CYCLE

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1 WOULD EXHAUST A LARGE PART OF THE PROGRAM FUNDS.
2 THAT'S WHAT HAPPENED THIS YEAR WHERE THE FIRST CYCLE
3 HAD ABOUT $120 MILLION WITH $160 MILLION DEPLOYED,
4 LEAVING 40 MILLION FOR THE SECOND CYCLE. AND WHAT
5 WE EXPECT IS THAT THAT'S GOING TO CREATE A MUCH
6 HIGHER DEMAND FOR THAT FIRST CYCLE OF THE FISCAL
7 YEAR EVERY YEAR.
8 SO IF WE WERE TO MOVE TO A SINGLE CYCLE
9 PER FISCAL YEAR, ALL APPLICATIONS WOULD COMPETE FOR
10 THE FULL PROGRAM BUDGET FOR THAT SINGLE CYCLE.
11 THERE WOULD BE A HIGHER VOLUME OF APPLICATIONS THAT
12 ARE REVIEWED TO ACCOUNT FOR THE FULL PROGRAM BUDGET.
13 AND THEN ANY UNFUNDED APPLICATIONS WOULD HAVE TO
14 RESUBMIT IN THE NEXT FISCAL YEAR APPLICATION CYCLE,
15 WHICH IS ROUGHLY SIX MONTHS.
16 AND THEN, LASTLY, IS THAT ANOTHER
17 IMPLICATION IS THAT IF WE HAVE A SINGLE CYCLE PER
18 YEAR, IT ALLOWS US TO BETTER LINE UP THE PROGRAM
19 UPDATES AND THE APPLICATION LIFE CYCLE TO OPTIMIZE
20 IT FOR THAT PARTICULAR PROGRAM.
21 SO THOSE ARE THE SORT OF IMPLICATIONS FOR
22 WHY WE ARE RECOMMENDING MOVING THE PDEV PROGRAM TO
23 ONE CYCLE PER FISCAL YEAR. AND I'LL PAUSE THERE,
24 AND THAT'S THE END OF THE PRESENTATION.
25 CHAIRMAN FISCHER-COLBRIE: OKAY. ANY

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QUESTIONS FOR SHYAM ON THE CYCLE?

SO I DON'T THINK WE'RE READY TO MAKE A MOTION BASED ON SOME OF THE QUESTIONS AND CONCERNS. I THINK WE NEED FURTHER QUALIFICATIONS AND CLARIFICATIONS ON SOME OF THE CRITICAL POINTS THAT ARE BROUGHT UP. BUT OBVIOUSLY WOULD OPEN THAT UP TO THE SCIENCE COMMITTEE FOR DISCUSSION, COMMENTS BY OUR TEAM MEMBERS, OR BY THE SCIENCE COMMITTEE ON THAT.

VICE CHAIR BONNEVILLE: I AGREE. I THINK THAT THERE'S A LOT OF STILL OUTSTANDING QUESTIONS THAT NEED TO BE ANSWERED. AND SO I WOULD EXPECT THAT -- I KNOW A LOT OF PEOPLE HAVE BEEN TAKING NOTES. SO THIS WOULD BE SOMETHING THAT GETS FULLY FLESHED OUT AND THEN COMES TO THE BOARD, FULL BOARD IN MARCH. AND IF IT CAN'T BE DONE BY THE FULL BOARD IN MARCH, THEN IT WOULD REALLY HAVE TO WAIT UNTIL JUNE. SO I WOULD JUST ENCOURAGE EVERYONE TO GO BACK THROUGH THE TRANSCRIPTS AND THE YOUTUBE AND MAKE SURE ALL THE QUESTIONS ARE ADDRESSED.

CHAIRMAN FISCHER-COLBRIE: YEAH. BECAUSE I THINK IT'S IMPORTANT TO GET THINGS DONE BY MARCH BECAUSE WE HAVE SIGNIFICANT ELEMENTS THAT NEED TO GET MODIFIED. AND I THINK THE CIRM TEAM HAS DONE A MARVELOUS JOB OF COMING UP WITH A MUCH, MUCH

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STRONGER APPROACH, NOT IMPLYING THAT THE LAST APPROACH WAS TERRIBLE. IT'S ALL A REFLECTION OF THE FACT THAT THIS IS AN ENTIRELY NEW PROCESS DRIVEN AROUND THE STRATEGIC ALLOCATION FRAMEWORK THAT HAS THE DIRECT GOAL OF GETTING GREATER LEVERAGE FOR THE INVESTMENTS THAT ARE BEING MADE. AND SO THE ENTIRE PROCESS OF THE ALLOCATION FRAMEWORK, THE APPROVAL PROCESS, AND WORKING THESE THINGS THROUGH, GETTING INFORMATION FROM WHAT WE'VE LEARNED WITH OUR FIRST SET OF MECHANISMS IS OF GREAT IMPORT AND VALUE. AND NOW WE JUST NEED TO TAKE THE NEXT STEP, BUT I THINK THE TIME CONSIDERATION IS IMPORTANT. SO I WOULD ADVOCATE THAT WE TRY TO GET SOMETHING ON THE CALENDAR AND GET IT DETERMINED SO THAT WE CAN GET AN APPROVAL FOR THE BOARD MEETING IN MARCH.

MR. TOCHER: MARK, THIS IS SCOTT OVER AT HQ. I THINK WHAT YOU'RE SUGGESTING IS THAT WE CONTINUE THIS MEETING TO A FUTURE DATE. AND FOR EVERYONE'S PLANNING PURPOSES, WE WILL POST THAT REVISION BY TOMORROW. BUT IT'S LOOKING LIKE THIS WOULD BE CONTINUED TO MARCH 19TH AT 2 P.M. SO PENCIL THAT IN, AND YOU CAN USE INK TOMORROW.

CHAIRMAN FISCHER-COLBRIE: GREAT. THAT SOUNDS GOOD, SCOTT.

ANY OTHER COMMENTS BY THE CIRM TEAM? AND,

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1 AGAIN, YOU'RE ALL TO BE COMMENDED BECAUSE YOU GUYS
2 HAVE PUT IN A MASSIVE AMOUNT OF WORK OVER AND ABOVE
3 YOUR ALREADY PRESSING DAY JOB. SO MUCH APPRECIATED.
4 BUT ANY COMMENTS BY THE CIRM TEAM?
5 DR. LEVITT: SO, MARK, I WOULD RECOMMEND
6 THAT THE CIRM -- THE CIRM TEAM TOOK COPIOUS NOTES.
7 THERE'S A TRANSCRIPT. IF THEY WANT CLARIFICATION ON
8 COMMENTS THAT WERE MADE IN THIS MEETING, I THINK
9 THEY SHOULD BE VERY AGGRESSIVE ABOUT CONTACTING THE
10 INDIVIDUAL WHO MADE THE COMMENT AND ASK FOR
11 CLARIFICATION. IF IT'S CLEAR, IT'S CLEAR. BUT I
12 THINK TO SAVE TIME, RIGHT, GET CLARIFICATION UP
13 FRONT. CONTACT THE MEMBERS OF THE SUBCOMMITTEE AND
14 SAY CAN YOU CLARIFY THIS BECAUSE WE DIDN'T QUITE GET
15 IT. AND THAT WOULD HELP DO THE MODIFICATIONS.
16 CHAIRMAN FISCHER-COLBRIE: THANKS, PAT.
17 DR. THOMAS: MARK, I'LL JUST WEIGH IN
18 WEIGH IN AND RESPOND. THANK YOU, PAT. AND JUST
19 THANK YOU TO EVERYBODY. THIS IS A VERY COMPLICATED
20 REVISION AS EVIDENCED BY THE LENGTH OF TIME THAT
21 WE'VE ALL SPENT TOGETHER HERE TODAY AND THE AMOUNT
22 OF WORK THAT'S GONE INTO GETTING US TO THIS POINT.
23 AND AS WITH ANYTHING, THERE ARE ALWAYS ISSUES THAT
24 COME UP THAT ARE VERY GOOD ISSUES REQUIRING A BIT
25 MORE THINKING TO GET TO THE FINISH LINE. AND SO

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WE'VE HAD A NUMBER OF THOSE TODAY. WE HEAR YOU. JUST AS WE HEARD YOU IN THE CONCERNS RAISED IN GENERAL WHICH RESULTED IN THIS VERY DIFFERENT APPROACH TO THE WHOLE PROCESS, WE WILL EVALUATE HOW BEST TO INTEGRATE YOUR COMMENTS INTO THE FINAL PRODUCT. IT WILL COME ON THE 19TH WITH THE GOAL OF GETTING SUBCOMMITTEE APPROVAL TO FORWARD THAT TO THE FULL BOARD. SO THANK YOU SO MUCH, EVERYBODY, FOR WHAT YOU'VE DONE. THANKS AGAIN TO THE TEAM FOR THE HERCULEAN EFFORT HERE, AND WE REALLY APPRECIATE IT. SO THANK YOU.

CHAIRMAN FISCHER-COLBRIE: GREAT JOB, GUYS. THANKS TO EVERYBODY ON THE COMMITTEE FOR THE CALL AND LOOK FORWARD TO TAKING NEXT STEPS SHORTLY.

MR. TOCHER: THANK YOU, MARK. THANK YOU, EVERYONE.

(THE MEETING WAS THEN CONCLUDED AT 12 28 P.M.)

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REPORTER'S CERTIFICATE

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE VIRTUAL PROCEEDINGS BEFORE THE SCIENCE SUBCOMMITTEE OF THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON MARCH 5, 2026, WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

BETH C. DRAIN, CA CSR 7152
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