BEFORE THE

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

REGULAR MEETING

LOCATION: AS INDICATED ON THE AGENDA

DATE: JULY 20, 2017
11 A.M.

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

FILE NO.: 2017-17

I N D E X

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JULY 20, 2017; 11 A.M.

CHAIRMAN THOMAS: WELCOME TO THE APPLICATION REVIEW SUBCOMMITTEE. SCOTT, WILL YOU PLEASE TAKE THE ROLL? MARIA IS ON VACATION, SO SCOTT IS STANDING IN FOR MARIA.

MR. TOCHER: ANNE-MARIE DULIEGE. DAVID HIGGINS. STEVE JUELSGAARD. SHERRY LANSING. KATHY LAPORTE.

MS. LAPORTE: HERE.

MR. TOCHER: THANK YOU. LAUREN MILLER. ADRIANA PADILLA. JOE PANETTA.

MR. PANETTA: HERE.

MR. TOCHER: THANK YOU. FRANCISCO PRIETO.

DR. PRIETO: HERE.

MR. TOCHER: THANK YOU. ROBERT QUINT.

DR. QUINT: HERE.

MR. TOCHER: THANK YOU. AL ROWLETT.

MR. ROWLETT: HERE.

MR. TOCHER: THANK YOU. JEFF SHEEHY.

SUPERVISOR SHEEHY: HERE.

MR. TOCHER: THANK YOU. OS STEWARD.

DR. STEWARD: HERE.

MR. TOCHER: JONATHAN THOMAS.

CHAIRMAN THOMAS: HERE.

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MR. TOCHER: THANK YOU. ART TORRES.

MR. TORRES: HERE.

MR. TOCHER: THANK YOU. DIANE WINOKUR.

MS. WINOKUR: HERE.

MR. TOCHER: HAS ANYONE JUST JOINED THE CALL? AND DO WE HAVE ANY OTHER BOARD MEMBERS ONLINE WHOSE NAME I HAVE NOT CALLED?

J.T., WE HAVE TEN. WE'RE JUST ONE SHORT OF A QUORUM, BUT I THINK WE CAN STILL GET STARTED WITH THE INTRODUCTION.

CHAIRMAN THOMAS: OKAY. GREAT. SO WE'RE GOING TO GO TO ITEM 3, WHICH IS CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO CLIN2: PARTNERING OPPORTUNITY FOR CLINICAL TRIAL STAGE PROJECTS. I'M GOING TO TURN THE MEETING OVER AT THIS POINT TO SUPERVISOR SHEEHY.

SUPERVISOR SHEEHY: THANK YOU, CHAIR THOMAS.

SO, DR. SAMBRANO, I THINK YOU HAVE A PRESENTATION TO START US OFF. WE'RE GOING TO START WITH THE CLIN.

DR. SAMBRANO: YES. I DO THANK YOU VERY MUCH. SO I'M SHOWING THE SLIDES ON THE WEBEX. THESE MAY HAVE BEEN DISTRIBUTED TO YOU AS WELL, AND YOU CAN VIEW THEM ON YOUR COMPUTER.

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JUST VERY BRIEFLY, THIS IS FOR THE CLINICAL STAGE PROGRAMS. WE'RE GOING TO CONSIDER ONE APPLICATION THAT IS RESPONDING TO THE CLIN2 CLINICAL TRIAL STAGE PROJECT.

A VERY BRIEF REMINDER OF THE SCORING SYSTEM THAT WE USE FOR OUR CLINICAL APPLICATIONS. WE USE A SYSTEM OF 1, 2, OR 3. ONE MEANING THAT THE APPLICATION HAS EXCEPTIONAL MERIT AND WARRANTS FUNDING. IF IT RECEIVES A SCORE OF 2, IT MEANS IT NEEDS IMPROVEMENT. AND A SCORE OF 3 MEANS THAT IT HAS FLAWS, AND THE PROJECT SHOULD NOT BE RESUBMITTED FOR AT LEAST SIX MONTHS.

THE PROJECT UNDER CONSIDERATION TODAY IS CLIN2-09894. THIS IS FOR A PHASE 3 CLINICAL TRIAL TO TREAT ALS. THE THERAPY IS AN AUTOLOGOUS MODIFIED MESENCHYMAL STEM CELL PRODUCT FOR PATIENTS WITH ALS. THEIR GOAL IS TO COMPLETE A PHASE 3 CLINICAL TRIAL TO ESTABLISH THE EFFICACY OF THIS CELL THERAPY IN PATIENTS WITH ALS. THE MAJOR PROPOSED ACTIVITIES UNDER THE PROPOSED APPLICATION INCLUDE THE MANUFACTURE OF THE CELL PRODUCT; ENROLLMENT OF ABOUT 200 PATIENTS FOR THE STUDY ACROSS SEVEN SITES, INCLUDING THE U.S., CALIFORNIA, AS WELL AS INTERNATIONAL SITES; AND, OF COURSE, THE TREATMENT AND CONDUCT OF THE CLINICAL TRIAL.

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THE FUNDS REQUESTED FOR THIS AWARD ARE 15.9 MILLION. THE APPLICANT IS PROVIDING AN EQUAL AMOUNT OF CO-FUNDING TO SUPPORT THE PROJECT ACTIVITIES.

AS YOU KNOW, THE REVIEW PROCESS INCLUDES AN INTERNAL BUDGET REVIEW WHICH THIS APPLICATION REVIEW PASSED. THE GRANTS WORKING GROUP GAVE THIS APPLICATION A SCORE OF 1, MEANING EXCEPTIONAL MERIT. AND THE NUMBER OF VOTES IN EACH CATEGORY, THERE WERE NINE MEMBERS OF THE GWG THAT GAVE IT A SCORE OF 1, THERE WERE THREE THAT GAVE IT A SCORE OF 2, AND NONE A SCORE OF 3. THE CIRM TEAM SUPPORTS THIS RECOMMENDATION FROM THE GWG FOR THE AWARD AMOUNT OF 15.9 MILLION. MR. SHEEHY.

SUPERVISOR SHEEHY: THANK YOU, DR. SAMBRANO.

DO I HAVE A MOTION TO ACCEPT THE TEAM'S RECOMMENDATION?

MR. TOCHER: EXCUSE ME, JEFF. THIS IS SCOTT. WE'RE STILL WAITING FOR ONE MORE MEMBER TO JOIN THE CALL TO ENSURE THAT WE HAVE A QUORUM. AND I BELIEVE AMY HAS REACHED SOMEONE. SHE JUST STEPPED OUT OF THE ROOM. SO IF WE COULD PAUSE FOR A MOMENT IF THERE'S ANY OTHER QUESTIONS ON IT PERHAPS.

SUPERVISOR SHEEHY: ANY DISCUSSION? DOES

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ANYONE WANT TO ASK ANY QUESTIONS OR ANYTHING?

MS. LAPORTE: I GUESS WHILE WE'RE WAITING, I'M CURIOUS. THE REVIEWERS' COMMENTS LAST GO-ROUND AND THIS GO-ROUND TALKED ABOUT THE CLINICAL TRIAL DESIGN AND THE LACK OF COMPARISON TO SYSTEMIC DISEASE. IS THERE A REASON THAT THAT WASN'T ABLE TO BE INCORPORATED?

DR. SAMBRANO: I THINK PART OF THAT WAS THE CLINICAL DESIGN THAT WAS ACCEPTED ALREADY BY THE FDA. THEY HAVE ALREADY GONE THROUGH A PHASE 2 CLINICAL TRIAL WITH THE SAME GENERAL DESIGN WHERE WHAT THEY ARE COMPARING TO IS THE EQUIVALENT OF A SALINE CONTROL RATHER THAN MSC. I THINK THEIR GOAL IS TO DEMONSTRATE THAT THEIR MODIFIED MSC'S SHOW EFFICACY RATHER THAN TRYING TO DEMONSTRATE THAT THERE IS EFFICACY ABOVE AND BEYOND JUST MSC'S ALONE.

SO THAT WAS A POINT OF DISCUSSION BY THE GWG, AND I THINK ULTIMATELY THEY FELT THAT THIS WAS SOMETHING THAT WAS NOT NECESSARY. AND SHOULD IT BECOME A QUESTION IN THE FUTURE IS SOMETHING THAT IF THEY DEMONSTRATE EFFICACY UNDER THIS TRIAL, THEY ALREADY HAVE A HEAD START AND CAN ALWAYS INCLUDE IT IN A LATER PHASE.

MS. LAPORTE: THANK YOU.

MR. TOCHER: DAVID HIGGINS SHOULD BE

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CALLING IN IN JUST A MOMENT.

(PAUSE IN PROCEEDINGS.)

MR. TOCHER: DAVID HIGGINS, HAVE YOU JOINED THE CALL?

DR. HIGGINS: YES, I HAVE. SORRY FOR BEING LATE.

MR. TOCHER: NO PROBLEM. JEFF, WE HAVE A QUORUM.

SUPERVISOR SHEEHY: GREAT. GREAT.

SO, DAVID, WE JUST HAD A DISCUSSION ABOUT THE CLIN2 APPLICATION MOVING FORWARD. DO YOU HAVE ANY QUESTIONS ABOUT THAT?

DR. HIGGINS: NO, I DO NOT.

SUPERVISOR SHEEHY: GREAT. SO AT THIS TIME DO I HAVE A MOTION TO --

MR. TORRES: SO MOVED.

SUPERVISOR SHEEHY: DO I HAVE A SECOND?

MR. ROWLETT: SECOND.

MS. LAPORTE: SECOND.

MR. TOCHER: WHO WAS THE SECOND PLEASE?

MR. ROWLETT: THIS IS AL ROWLETT.

MR. TOCHER: THANK YOU, AL.

SUPERVISOR SHEEHY: IS THERE ANY PUBLIC COMMENT AT ANY OF THE SITES? SO, AMY OR SCOTT, I GUESS, SCOTT, YOU'RE CALLING THE CALL THE ROLL

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TODAY. COULD YOU CALL THE ROLL PLEASE.

MR. TOCHER: DAVID HIGGINS.

DR. HIGGINS: YES.

MR TOCHER: STEVE JUELSGAARD. KATHY LAPORTE.

MS. LAPORTE: YES.

MR. TOCHER: JOE PANETTA.

MR. PANETTA: YES.

MR. TOCHER: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MR. TOCHER: ROBERT QUINT.

DR. QUINT: YES.

MR. TOCHER: AL ROWLETT.

MR. ROWLETT: YES.

MR. TOCHER: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MR. TOCHER: ART TORRES.

MR. TORRES: AYE.

MR. TOCHER: JEFF SHEEHY.

MR. SHEEHY: YES.

MR. TOCHER: GREAT. THAT MOTION CARRIES.

SUPERVISOR SHEEHY: GIL, COULD YOU TAKE US INTO THE TRANSLATION ROUND, PLEASE?

DR. SAMBRANO: ABSOLUTELY.

SO THE TRANSLATION PROGRAM IS ONE THAT WE

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CARRY OUT THREE REVIEWS PER YEAR. AND THIS FITS RIGHT IN THE CENTER OF OUR FUNDING OPPORTUNITIES BETWEEN DISCOVERY AND CLINICAL. IT TAKES PROJECTS THAT HAVE IDENTIFIED A CLEAR PRODUCT CANDIDATE TO TAKE THROUGH THE TRANSLATIONAL PHASE AND GET TO WHERE THEY CAN CONDUCT A PRE-IND MEETING OR EQUIVALENT IN ORDER TO MOVE THE PRODUCT INTO THE CLINIC.

SO THAT IS THE OVERALL OBJECTIVE OF THE TRANSLATIONAL RESEARCH PROGRAM. IT WILL COVER APPROXIMATELY TWO YEARS OF WORK. THERE ARE SEVERAL PRODUCT TYPES THAT QUALIFY UNDER THIS PROGRAM, INCLUDING A THERAPY, SUCH AS A CELL THERAPY OR A DRUG, IT ALSO INCLUDES DIAGNOSTICS, MEDICAL DEVICES, AND TOOLS. AND ONE OF THE THINGS THAT WE LOOK FOR IN THESE PROPOSALS THAT ARE COMING IN IS THAT THEY ARE AT THE APPROPRIATE STAGE OF READINESS, MEANING THAT THEY HAVE INDEED IDENTIFIED A SINGLE CANDIDATE THAT HAS DISEASE-MODIFYING ACTIVITY, AS AN EXAMPLE, FOR SOMETHING THAT IS A THERAPEUTIC, OR FOR OTHER DIAGNOSTICS OR TOOLS WITH A PROTOTYPE WITH A PROOF OF CONCEPT.

AND THEN THE OUTCOME AS PROPOSED UNDER THE APPLICATION SHOULD LEAD WITHIN A TIME PERIOD TO THE COMPLETION OF A PRE-IND MEETING OR IDE MEETING; OR,

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IF IT'S A TOOL, TO MANUFACTURE FOR COMMERCIALIZATION.

THE REVIEW CRITERIA THAT ARE UTILIZED BY GWG FOR THIS PROGRAM ARE THE FOUR KEY QUESTIONS: WHETHER THE PROJECT HOLDS THE NECESSARY SIGNIFICANCE AND POTENTIAL FOR IMPACT, THAT IS, THE OVERALL VALUE THAT IT BRINGS; WHETHER THE RATIONALE IS SOUND, MEANING IS IT A PROJECT THAT MAKES SENSE AND HAS THE SUPPORTING DATA; IS IT WELL PLANNED AND DESIGNED; AND IS IT FEASIBLE, MEANING DO THEY HAVE THE APPROPRIATE RESOURCES, THE RIGHT PERSONNEL, AND HAVE ASSEMBLED A GOOD TEAM TO CARRY OUT THE PROJECT.

THE SCORING SYSTEM HERE IS DIFFERENT FROM CLIN. WE USE A SCORING SYSTEM FROM 1 TO 100. SO A SCORE THAT FALLS IN THE RANGE OF 85 TO 100 MEANS THAT IT'S AN APPLICATION THAT'S RECOMMENDED FOR FUNDING. IF IT RECEIVES A SCORE OF 1 TO 84 MEANS IT IS NOT RECOMMENDED FOR FUNDING. IT IS THE MEDIAN OF ALL THE INDIVIDUAL GWG SCORES FOR THAT APPLICATION THAT DETERMINES THE FINAL SCORE.

FOR THIS ROUND OF TRANSLATION, THERE WERE NINE APPLICATIONS THAT WERE REVIEWED. THERE WAS ONE THAT WAS RECOMMENDED FOR A TOTAL AMOUNT OF 5.8 MILLION THAT IS BEING REQUESTED. AND I'M JUST GOING TO GIVE YOU AN OVERVIEW OF THAT ONE APPLICATION JUST

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TO GIVE YOU HIGHLIGHTS WHAT THAT'S ABOUT.

IT'S ENTITLED "DEVELOPMENT OF ROR1 CAR-T CELLS TO TARGET CANCER STEM CELLS IN ADVANCED MALIGNANCIES." SO THOSE MALIGNANCIES INCLUDE THINGS LIKE PANCREATIC CANCER, BREAST CANCER, CLL, AND HEAD AND NECK SQUAMOUS CELL CARCINOMA.

AND THEIR GOAL IS TO TRY TO ASSESS FROM AMONG THESE DIFFERENT INDICATIONS OR CANCER INDICATIONS WHICH MAY BE THE BEST FIT FOR DEVELOPING THIS ROR1 CAR-T CELL THERAPY. AND IN DOING SO, GET TO THE POINT WHERE THEY CAN HAVE A PRE-IND MEETING WITH THE FDA WITH THAT INDICATION IN ORDER TO MOVE FORWARD INTO THE CLINIC.

SO THAT'S IT FROM ME. IF THERE ARE ANY QUESTIONS, HAPPY TO ADDRESS THEM. MR. SHEEHY.

SUPERVISOR SHEEHY: SO, FIRST, COULD I GET A MOTION TO ACCEPT THE TEAM'S RECOMMENDATION?

DR. PRIETO: SO MOVED.

SUPERVISOR SHEEHY: DR. PRIETO. DO I HAVE A SECOND?

MS. LAPORTE: SECOND.

SUPERVISOR SHEEHY: IS THERE ANY DISCUSSION ABOUT THIS OR EVEN ANY OTHER GRANTS? IS THERE ANY PUBLIC COMMENT? SO, SCOTT, COULD YOU CALL THE ROLL PLEASE.

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MR. TOCHER: HIGGINS.

DR. HIGGINS: YES.

MR. TOCHER: JUELSGAARD.

MR. JUELSGAARD: YES.

MR. TOCHER: LAPORTE.

MS. LAPORTE: YES.

MR. TOCHER: PANETTA.

MR. PANETTA: YES.

MR. TOCHER: PRIETO.

DR. PRIETO: AYE.

MR. TOCHER: QUINT.

DR. QUINT: YES.

MR. TOCHER: ROWLETT.

MR. ROWLETT: YES.

MR. TOCHER: STEWARD.

DR. STEWARD: YES.

MR. TOCHER: THOMAS.

CHAIRMAN THOMAS: YES.

MR. TOCHER: TORRES.

MR. TORRES: AYE.

MR. TOCHER: WINOKUR.

MS. WINOKUR: YES.

MR. TOCHER: SHEEHY.

SUPERVISOR SHEEHY: YES.

MR. TOCHER: THANK YOU. THE MOTION

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CARRIES.

SUPERVISOR SHEEHY: CHAIRMAN THOMAS, THIS CONCLUDES THE BUSINESS OF THE APPLICATION REVIEW SUBCOMMITTEE.

MR. TOCHER: EXCUSE ME. SORRY. THERE'S JUST ONE LAST OMNIBUS MOTION TO NOT FUND ANY OF THE REMAINING APPLICATIONS IN THE REMAINING TIER.

SUPERVISOR SHEEHY: I APOLOGIZE. COULD I GET SOMEONE TO MAKE THAT MOTION?

MR. TORRES: SO MOVED.

DR. PRIETO: SECOND.

SUPERVISOR SHEEHY: I DO BELIEVE IF ANYONE HAS CONFLICTS WITH ANY OF THE REMAINING APPLICATIONS, THERE'S THE FORM TO RESPOND YES, EXCEPT FOR THOSE WITH WHICH I'M IN CONFLICT. SO COULD WE CALL THE ROLL, SCOTT. OH, NO. PUBLIC COMMENT. CALL THE ROLL PLEASE.

MR. TOCHER: HIGGINS.

DR. HIGGINS: YES.

MR. TOCHER: JUELSGAARD.

MR. JUELSGAARD: YES.

MR. TOCHER: LAPORTE.

MS. LAPORTE: YES.

MR. TOCHER: PANETTA.

MR. PANETTA: YES.

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MR. TOCHER: PRIETO.

DR. PRIETO: AYE.

MR. TOCHER: QUINT.

DR. QUINT: YES.

MR. TOCHER: ROWLETT.

MR. ROWLETT: YES.

MR. TOCHER: STEWARD.

DR. STEWARD: YES, EXCEPT FOR THOSE WITH WHICH I'M IN CONFLICT. AND I BELIEVE I ALSO NEEDED TO VOTE IN THAT MANNER ON THE PREVIOUS MOTION.

MR. TOCHER: ACTUALLY YOU WERE OKAY ON THE PREVIOUS MOTION.

DR. STEWARD: OKAY. THANK YOU.

MR. TOCHER: THOMAS.

CHAIRMAN THOMAS: YES.

MR. TOCHER: TORRES.

MR. TORRES: AYE.

MR. TOCHER: WINOKUR.

MS. WINOKUR: YES.

MR. TOCHER: AND SHEEHY.

MR. SHEEHY: YES.

MR. TOCHER: GREAT. THANK YOU. THAT MOTION CARRIES.

SUPERVISOR SHEEHY: CHAIRMAN THOMAS, THE WORK OF THE APPLICATION REVIEW SUBCOMMITTEE IS

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CONCLUDED.

CHAIRMAN THOMAS: THANK YOU, MR. SUPERVISOR. DO WE HAVE ANY PUBLIC COMMENT IN GENERAL ON ANY TOPIC FROM ANY OF THE LOCATIONS? HEARING NONE, I WOULD ENTERTAIN A MOTION TO ADJOURN.

SUPERVISOR SHEEHY: SO MOVED.

CHAIRMAN THOMAS: IS THERE A SECOND?

MS. LAPORTE: SECOND.

CHAIRMAN THOMAS: ALL THOSE IN FAVOR? VERY GOOD. THAT CONCLUDES TODAY'S BUSINESS. THANK YOU VERY MUCH, EVERYBODY. HAVE A GREAT NEXT MONTH AND WE'LL TALK TO YOU IN AUGUST.

(THE MEETING WAS THEN CONCLUDED AT 11:26 A.M.)

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REPORTER'S CERTIFICATE

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE TELEPHONIC PROCEEDINGS BEFORE THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE AND THE APPLICATION REVIEW SUBCOMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON JULY 20, 2017, WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

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