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: NOVEMBER 30, 2017
11 A.M.

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

FILE NO.: 2017-25

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

I N D E X

ITEM DESCRIPTION	PAGE NO.
OPEN SESSION	
1. CALL TO ORDER.	3
2. ROLL CALL.	3
3. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO TRAN: TRANSLATIONAL REVIEW.	5
4. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO DISC1: INCEPTION REVIEW.	12
CLOSED SESSION	NONE
5. DISCUSSION OF CONFIDENTIAL INTELLECTUAL PROPERTY OR WORK PRODUCT, PREPUBLICATION DATA, FINANCIAL INFORMATION, CONFIDENTIAL SCIENTIFIC RESEARCH OR DATA, AND OTHER PROPRIETARY INFORMATION RELATING TO APPLICATIONS SUBMITTED IN RESPONSE TO TRAN: TRANSLATIONAL REVIEW AND DISC1: INCEPTION REVIEW (HEALTH & SAFETY CODE 125290.30(F) (3) (B) AND (C)).	
OPEN SESSION	
6. PUBLIC COMMENT.	NONE
7. ADJOURNMENT.	26

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

THURSDAY, NOVEMBER 30, 2017; 11 A.M.

CHAIRMAN THOMAS: THANK YOU. I'D LIKE TO WELCOME EVERYBODY TO THE REGULAR MEETING OF THE ICOC AND THE APPLICATION REVIEW SUBCOMMITTEE FOR NOVEMBER 2017. DAVID HIGGINS AND I ARE DOING THIS MEETING FROM THE SANFORD CONSORTIUM, A BEAUTIFUL SPOT DOWN IN LA JOLLA. MARIA, CAN YOU PLEASE CALL THE ROLL.

MS. BONNEVILLE: ANNEMARIE DULIEGE. DAVID HIGGINS.

DR. HIGGINS: HERE.

MS. BONNEVILLE: STEVE JUELSGAARD.

DR. JUELSGAARD: HERE.

MS. BONNEVILLE: SHERRY LANSING. DAVE MARTIN.

DR. MARTIN: HERE.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: HERE.

MS. BONNEVILLE: ADRIANA PADILLA. JOE PANETTA. FRANCISCO PRIETO. ROBERT QUINT. AL ROWLETT.

MR. ROWLETT: HERE.

MS. BONNEVILLE: JEFF SHEEHY. OS STEWARD.

DR. STEWARD: HERE.

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: HERE.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: HERE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: HERE.
MS. BONNEVILLE: THANK YOU.
WE'RE WAITING FOR A COUPLE OF BOARD MEMBERS TO JOIN.
DR. PADILLA: I JUST CALLED IN AGAIN.
MS. BONNEVILLE: ARE THERE ANY MEMBERS OF THE BOARD AT YOUR LOCATION?
DR. PADILLA: NO.
CHAIRMAN THOMAS: WE HAVE ONE MEMBER OF THE PUBLIC HERE IN LA JOLLA.
MS. BONNEVILLE: ARE THERE OTHER BOARD MEMBERS ON THE LINE THAT I'VE NOT CALLED? OKAY. THANK YOU. GO AHEAD, J.T.
CHAIRMAN THOMAS: OKAY. THANK YOU, EVERYBODY. SUPERVISOR SHEEHY WAS NOT ABLE TO MAKE IT TODAY --
MS. BONNEVILLE: HE'S GOING TO JOIN LATE; BUT IF YOU COULD FILL IN FOR HIM UNTIL HE JOINS, THAT WOULD BE FANTASTIC.
CHAIRMAN THOMAS: SO I WILL PINCH HIT

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

UNTIL HE GETS HERE.

WE'LL GO TO ITEM NO. 3 ON THE AGENDA, CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO THE TRAN OR TRANSLATIONAL REVIEW. WE HAVE A PRESENTATION, DR. SAMBRANO.

DR. SAMBRANO: THANK YOU VERY MUCH, MR. CHAIRMAN. SO AS YOU KNOW, WE HAVE RECURRING FUNDING OPPORTUNITIES AVAILABLE ACROSS THE SPECTRUM OF THE PIPELINE THAT WE FUND RANGING FROM DISCOVERY TO CLINICAL TRIALS. SO THE TRANSLATION PROGRAM FITS RIGHT IN THE MIDDLE. ITS GOAL IS TO SUPPORT PROMISING STEM CELL-BASED PROJECTS THAT WOULD ACCELERATE COMPLETION OF PROJECTS THAT ARE AT THAT TRANSLATIONAL STAGE AND ADVANCE THEM ON TO THE BEGINNINGS OF A CLINICAL STUDY OR BROAD END USE.

WE SUPPORT THE DEVELOPMENT OF PRODUCTS ACROSS DIFFERENT CATEGORIES INCLUDING THERAPEUTICS, WHICH IS THE MOST POPULAR AND ONE THAT NORMALLY COMES TO MIND AS A DRUG OR A THERAPY, BUT WE ALSO SUPPORT DIAGNOSTICS, MEDICAL DEVICES, AND TOOLS FOR DEVELOPMENT. AND FOR EACH OF THESE THERE ARE DIFFERENT CRITERIA IN TERMS OF WHAT IS REQUIRED TO GET TO THAT TRANSLATIONAL STAGE. BUT GENERALLY SPEAKING, WHAT WE EXPECT FOR A PROJECT COMING INTO THE TRANSLATION PROGRAM IS THAT IF, FOR EXAMPLE, IT

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

IS A THERAPEUTIC, THAT IT HAS A SINGLE DEVELOPMENT CANDIDATE THAT HAS DEMONSTRATED DISEASE MODIFYING ACTIVITY. IF IT'S ONE OF THE OTHER TYPES OF PRODUCTS, THAT THEY HAVE A PROTOTYPE WHERE THEY'VE SHOWN A PROOF OF CONCEPT. SO THOSE TYPES OF PROJECTS ARE READY FOR ENTERING INTO THE TRANSLATIONAL PHASE. AND THE EXPECTED OUTCOME, ONCE THEY COMPLETE THOSE TRANSLATIONAL STUDIES, GENERALLY OVER A TWO-YEAR PERIOD, IS TO CONDUCT A PRE-IND MEETING IF IT'S A THERAPEUTIC OR ANOTHER PRESUBMISSION MEETING WITH THE FDA. IF IT HAPPENS TO BE A TOOL, THERE WE WANT TO SEE THEM TRANSFER TO MANUFACTURING FOR COMMERCIALIZATION OF THAT PRODUCT. SO THAT'S A GENERAL BIG PICTURE OF WHAT IT IS THE TRANSLATION PROGRAM COVERS.

THE REVIEW CRITERIA THAT ARE UTILIZED BY THE GRANTS WORKING GROUP TO ASSESS THE MERIT OF THESE APPLICATIONS FALLS INTO THESE FOUR BASIC QUESTIONS. THESE ARE THE ONES THAT OFTEN ARE USED FOR MOST OF OUR REVIEWS. THE FIRST IS DOES THE PROJECT HOLD THE NECESSARY SIGNIFICANCE AND POTENTIAL FOR IMPACT; THAT IS, TRYING TO ASSESS THE OVERALL VALUE THAT THE PROJECT BRINGS. IS THE RATIONALE SOUND, MEANING IS IT SOMETHING THAT MAKES SENSE? DO THEY HAVE DATA TO DEMONSTRATE THAT THEY

HAVE WHAT IS NECESSARY TO MOVE FORWARD? IS THE PROJECT WELL PLANNED AND DESIGNED? AND IS THE PROJECT FEASIBLE, MEANING DO THEY HAVE A QUALIFIED TEAM AND ALL THE RESOURCES AVAILABLE TO CONDUCT THE PROJECT WITHIN THE TIMELINE THAT IS PROPOSED.

THE SCORING SYSTEM THAT'S USED IN THE TRANSLATION PROGRAM IS THE SAME AS FOR ALL DISCOVERY AND TRAN PROGRAMS, A SCALE OF ONE TO A HUNDRED. A SCORE OF 85 TO A HUNDRED MEANS THAT IT'S RECOMMENDED FOR FUNDING IF FUNDS ARE AVAILABLE. ANYTHING THAT RECEIVES A SCORE OF 1 TO 84 MEANS IT'S NOT RECOMMENDED FOR FUNDING. WE USE THE MEDIAN FROM ALL THE INDIVIDUAL GWG SCORES TO DETERMINE THE FINAL SCORE.

(INTERRUPTION IN PROCEEDINGS.)

DR. SAMBRANO: SO THAT'S THE SCORING SYSTEM THAT WE USE FOR THIS PARTICULAR CYCLE OF THE TRAN PROGRAM. WE HAD 14 APPLICATIONS THAT WERE REVIEWED. THERE WERE THREE THAT RECEIVED A FUND RECOMMENDATION. THE TOTAL AMOUNT THAT IS REQUESTED FROM THE COMBINED THREE APPLICANTS WOULD TOTAL TO 13.4 MILLION. THERE ARE, AT LEAST IN THE ANNUAL ALLOCATION FOR THIS PROGRAM, SUFFICIENT FUNDS TO COVER THAT REQUEST.

SO I'M GOING TO JUST BRIEFLY REVIEW THE

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

THREE APPLICATIONS THAT ARE RECOMMENDED JUST TO GIVE YOU AN OVERVIEW OF WHAT THESE ARE ABOUT.

THE FIRST APPLICATION IS TRAN1-10416. SO THIS IS FOR A THERAPEUTIC. IT'S ENTITLED "DBCT GENETICALLY CORRECTED INDUCED PLURIPOTENT CELL-DERIVED EPITHELIAL SHEETS FOR DEFINITIVE TREATMENT OF DYSTROPHIC EPIDERMOLYSIS BULLOSA."

SO THIS IS A RARE DISEASE THAT AFFECTS CHILDREN AND ADULTS, WHICH CAUSES WOUNDING AND BLISTERING ON THE SKIN AS A RESULT OF A LACK OF COLLAGEN 7 IN THEIR KERATINOCYTES.

SO WHAT THIS WOULD DO IS THE PRODUCT IS A GENE-MODIFIED CELL THERAPY WHERE THEY WOULD TAKE CELLS FROM THE PATIENT, CONVERT THEM INTO IPSC'S, DO THE GENE CORRECTION, AND DIFFERENTIATE THEM INTO KERATINOCYTES TO CREATE SHEETS THAT WOULD BE APPLIED TO THE WOUND.

THE NEXT APPLICATION IS TRAN1-10587. THIS ONE IS ENTITLED "HUMAN EMBRYONIC STEM CELL-DERIVED NATURAL KILLER CELLS FOR CANCER TREATMENT." THE GOAL HERE IS TO CREATE AN OFF-THE-SHELF CELL THERAPY FOR TREATING PATIENTS THAT HAVE FAILED DIFFICULT TREATMENT FOR ACUTE MYELOID LEUKEMIA. WHAT THEY WOULD GENERATE WOULD BE FROM HUMAN EMBRYONIC STEM CELLS NATURAL KILLER CELLS THAT UTILIZE THE INNATE

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

IMMUNE SYSTEM TO TARGET CANCER CELLS.

THE NEXT APPLICATION IS TRAN1-10540 ENTITLED "SLICING MODULATOR TARGETING CANCER STEM CELLS IN ACUTE MYELOID LEUKEMIA." THIS IS, AGAIN, THE SAME DISEASE INDICATION, PATIENTS THAT HAVE REFRACTORY OR RELAPSING ACUTE MYELOID LEUKEMIA. THE PRODUCT HERE IS A SMALL MOLECULE THERAPEUTIC WHICH HAPPENS TO BE AN RNA-SPLICED MODULATOR INHIBITOR. WHAT THAT DOES IS THAT A CANCER STEM CELL IS BELIEVED TO HAVE ABERRANT SPLICING OF THEIR SURVIVAL GENES, SO IT UPSETS THE BALANCE IN THOSE CELLS SO THAT THEY BECOME RESISTANT TO CHEMOTHERAPY AND OTHER AGENTS. USING THIS INHIBITOR RESTORES THAT BALANCE AND ALLOWS THE CELLS TO BECOME SIMILAR TO NORMAL HEMATOPOIETIC STEM CELLS AND BECOME SUSCEPTIBLE TO CHEMOTHERAPY.

SO THOSE ARE THE THREE PROGRAMS RECOMMENDED FOR FUNDING. HAPPY TO TAKE ANY QUESTIONS.

CHAIRMAN THOMAS: ANY QUESTIONS FROM MEMBERS OF THE BOARD?

DR. MARTIN: MY QUESTION ON THE LAST ONE IS WHETHER THE SMALL MOLECULE IS BEING TARGETED TO THE CANCER STEM CELLS, OR IT'S SIMPLY TARGETING THE LEUKEMIC CELLS IN TOTO.

DR. SAMBRANO: SURE, DR. MARTIN. THE MOLECULE TARGETS CANCER STEM CELLS BY VIRTUE OF THE FACT THAT THOSE ARE THE ONES THAT HAVE THE ABERRANT SPLICING. AND SO ALTHOUGH IT MAY IMPACT OTHER CELL TYPES, IT IS THE CANCER STEM CELLS THAT ARE BEING AFFECTED BY THIS INHIBITOR.

DR. MARTIN: THANK YOU.

CHAIRMAN THOMAS: QUESTIONS FROM MEMBERS OF THE BOARD? OKAY. HEARING NONE, ARE THERE ANY OF THE PROJECTS THAT ANYBODY WOULD LIKE TO MOVE FROM TIER II UP TO TIER I?

ARE THERE ANY PROJECTS IN TIER I THAT ANYBODY WOULD LIKE TO MOVE DOWN TO TIER II? OKAY. HEARING NONE, I WOULD LIKE TO ENTERTAIN A MOTION THAT WE APPROVE ALL APPLICATIONS IN TIER I AND NOT APPROVE THOSE IN TIER II.

MR. ROWLETT: SO MOVED.

CHAIRMAN THOMAS: THANK YOU, AL. IS THERE A SECOND?

DR. HIGGINS: SECOND.

CHAIRMAN THOMAS: SECONDED BY DAVID. IT'S BEEN MOVED AND SECONDED. DO WE HAVE ANY PUBLIC COMMENT ON THIS MOTION? HEARING NONE, MARIA, WILL YOU PLEASE CALL THE ROLL.

MR. TOCHER: I JUST WANT TO REMIND

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

MEMBERS, FOR THOSE WHO HAVE A CONFLICT WITH REGARD TO ANY APPLICATION IN THIS SET, YOU SHOULD REPLY AYE OR NAY EXCEPT WITH RESPECT TO THOSE APPLICATIONS WITH WHICH I HAVE A CONFLICT.

MS. BONNEVILLE: ANNEMARIE DULIEGE. DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

DR. JUELSGAARD: YES.

MS. BONNEVILLE: SHERRY LANSING. DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: YES.

MS. BONNEVILLE: ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: JOE PANETTA. FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT. AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY. OS STEWARD.

DR. STEWARD: YES.

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

MR. TOCHER: OS, JUST REMIND YOU THAT YOU ARE IN CONFLICT WITH ONE OF THESE.

DR. STEWARD: YES, EXCEPT FOR THOSE WITH WHICH I'M IN CONFLICT.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: YES.

MS. BONNEVILLE: MOTION CARRIES.

CHAIRMAN THOMAS: THANK YOU, MARIA.

ON TO ITEM NO. 4 ON THE AGENDA, WHICH IS CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO THE DISC INCEPTION REVIEW. WE HAVE A PRESENTATION BY DR. SAMBRANO.

DR. SAMBRANO: MR. CHAIRMAN, SO, AGAIN, THIS IS THE SAME DIAGRAM I SHOWED BEFORE REGARDING OUR FUNDING OPPORTUNITIES SIMPLY TO POINT OUT WHERE THE INCEPTION PROGRAM FITS. THIS IS AT THE VERY BEGINNING OR ONSET OF OUR FUNDING OPPORTUNITIES IN THE DISCOVERY PROGRAM WHICH IS INTENDED TO SPAWN THE DEVELOPMENT OF GREAT NEW IDEAS. AND SO THAT IS ESSENTIALLY WHAT WE'RE LOOKING FOR IN THIS PROGRAM.

THE EMPHASIS OF THE GRANTS WORKING GROUP

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

REVIEW, THAT IS, THE GUIDANCE THAT WE GAVE TO OUR REVIEWERS, WAS THAT WE'RE LOOKING FOR GREAT NEW IDEAS WITH THE POTENTIAL TO RESULT IN A TRANSLATABLE HUMAN STEM PROGENITOR CELL-BASED PRODUCT OR TECHNOLOGY EVENTUALLY DOWN THE ROAD. WE EMPHASIZE THAT THE IDEAS WITH A SOUND SCIENTIFIC RATIONALE ARE IMPORTANT. IT IS ESSENTIAL WITHIN THE CRITERIA, BUT ALSO THAT PRELIMINARY DATA ARE NOT REQUIRED OR EXPECTED AT THIS STAGE. THIS IS THE VERY BEGINNING OF A PROJECT IDEA; AND SO, THEREFORE, PRELIMINARY DATA IS NOT NECESSARY HERE. AND THIS IS A HIGH RISK, HIGH REWARD PROGRAM. SO WE ARE CERTAINLY WILLING TO TAKE A RISK ON THE POTENTIAL DEVELOPMENT OF AN IDEA.

OF COURSE, THE GOAL HERE IS TO PROVIDE 150 K TO TEST THAT IDEA AND GENERATE DATA THAT WOULD ALLOW THE APPLICANT TO COMPETE FOR A LARGER, MORE SUBSTANTIAL FUNDING OPPORTUNITY WHETHER IT BE WITH CIRM OR ANOTHER FUNDER.

THE SCORING SYSTEM IS THE SAME AS WE USE WITH TRAN, MEANING 85 TO A HUNDRED MEANING IT'S MERITORIOUS; 1 TO 84, THAT REVIEWERS FEEL IT WAS NOT SUFFICIENT TO BE RECOMMENDED FOR FUNDING.

FOR THIS CYCLE OF DISC1, WE HAD 41 APPLICATIONS THAT WERE REVIEWED. THERE WERE 13 THAT

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

RECEIVED A SCORE BETWEEN 85 AND A HUNDRED. AND THE TOTAL AMOUNT FOR THOSE, TO FUND THOSE 13 PROGRAMS, IS $2.87 MILLION APPROXIMATELY.

MR. CHAIRMAN, I'M HAPPY TO GO THROUGH JUST A VERY BRIEF OVERVIEW OF EACH OF THESE, THERE ARE 13, OR I CAN ADDRESS SPECIFIC QUESTIONS, IF THERE ARE ANY, OF ANY OF THE APPLICATIONS AS THE GROUP DESIRES.

CHAIRMAN THOMAS: I WOULD GUESS -- THANK YOU FOR THE PRESENTATION, DR. SAMBRANO. I WOULD GUESS THAT MEMBERS OF THE SUBCOMMITTEE HERE HAVE HAD A CHANCE TO REVIEW THE SLIDES. SO I'LL JUST ASK ARE THERE ANY QUESTIONS FROM ANY MEMBERS ABOUT ANY OF THE SPECIFIC PROJECTS LISTED IN THE PRESENTATION? OKAY.

HEARING NONE, DO WE HAVE ANY MOTIONS BY A MEMBER OF THE SUBCOMMITTEE TO MOVE ANY PROJECTS FROM TIER II TO TIER I?

DR. HIGGINS: I WOULD LIKE TO MAKE A MOTION A MOVE DISC-10674, A NEW PHENOTYPIC SCREENING PLATFORM, FROM THE UNFUNDED TO THE FUNDED CATEGORY.

MR. TORRES: COULD YOU EXPLAIN THE RATIONALE PLEASE?

DR. HIGGINS: I CAN EXPLAIN IT NOW OR AFTER THE MOTION IS SECONDED.

MR. TORRES: THANK YOU.

CHAIRMAN THOMAS: TO GET THE DISCUSSION GOING HERE, I'LL SECOND THE MOTION.

DR. HIGGINS: I'D LIKE TO SPEAK. I ACTUALLY WANT TO EXPLAIN IN AS MUCH A NONSCIENTIFIC WAY AS A SCIENTIFIC WAY BECAUSE DR. NIENABER CAN DO THAT BETTER THAN I CAN. BUT PARKINSON'S DISEASE HAS BEEN DESCRIBED, WAS FIRST DESCRIBED OVER 200 YEARS AGO, BUT THE MOST COMMON DRUG THAT WE STILL TAKE TODAY IS OVER 70 YEARS OLD. THERE ARE SIMPLY NOTHING BUT REFORMULATIONS OF LEVODOPA AS SO-CALLED NEW DRUGS. SO WE'RE IN DESPERATE NEED.

SO I'M NOT AN EXPERT IN THE TECHNOLOGY, BUT I AM AN EXPERT IN PARKINSON'S. I KNOW WHAT IT MEANS TO NEED THESE NEW DRUGS. I DON'T KNOW IF YOU GUYS KNOW, BUT I'M A FOURTH GENERATION PARKINSON'S IN MY FAMILY. AND HERE WE ARE IN 2017, AND I TAKE THE SAME DRUG THAT MY GRANDMOTHER TOOK IN THE 1960S.

SO THIS PROPOSAL IS TO SET UP A SCREEN TO HELP IDENTIFY NEW DRUGS FOR PARKINSON'S IN A NUMBER OF WAYS, WHICH DR. NIENABER CAN DESCRIBE. BUT IT SEEMS TO ME LIKE IT FITS PERFECTLY WITH CIRM'S CRITERIA THAT THEY'RE TRYING TO ACHIEVE, THAT GIL JUST DESCRIBED. IT'S A SMALL AMOUNT OF MONEY, SORT OF LIKE SEED MONEY, FOR GREAT IDEAS THAT QUALIFIED

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

SCIENTISTS CAN DO. THAT REQUIRES PRELIMINARY DATA AND ENCOURAGES OUT-OF-THE-BOX THINKING, ETC., ETC. I THINK THAT THIS PROPOSAL FITS EVERY ONE OF THOSE CRITERIA.

AND I BELIEVE THAT THIS GRANT REPRESENTS EXACTLY THE KIND OF WORK THAT THE CIRM INCEPTION RFA IS DESIGNED TO PROMOTE. SO I'M ASKING THE COMMITTEE TO RECOMMEND FUNDING OF THIS GRANT, AND I'D BE HAPPY TO ANSWER ANY QUESTIONS FROM MY PERSPECTIVE.

CHAIRMAN THOMAS: SENATOR TORRES, DID THAT ANSWER THE QUESTION?

MR. TORRES: YES. THANK YOU SO MUCH.

CHAIRMAN THOMAS: ARE THERE QUESTIONS OR COMMENTS FROM MEMBERS OF THE BOARD ON THIS MOTION?

DR. PRIETO: WHAT WAS THE SCORING ON THIS APPLICATION?

CHAIRMAN THOMAS: I BELIEVE, DR. SAMBRANO, CORRECT ME IF I'M WRONG, I BELIEVE IT GOT AN 80.

DR. SAMBRANO: THAT'S CORRECT.

DR. PRIETO: WERE THERE ANY SPECIFIC CONCERNS RAISED IN THE DISCUSSION? I DON'T RECALL THE SPECIFICS OF THIS ONE.

DR. SAMBRANO: YES. SO THERE WERE SOME CONCERNS. I THINK THE LARGEST OR THE MAJOR CONCERN WAS RELATED TO VALIDATING THE TECHNOLOGY. SO WHAT

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

REVIEWERS WERE LOOKING FOR WAS DEMONSTRATION THAT THIS SCREENING TECHNOLOGY WOULD ALLOW YOU TO IDENTIFY A POTENTIAL, NOT ONLY A THERAPEUTIC TARGET, BUT ALSO A SMALL MOLECULE DRUG THAT WOULD ACT ON THAT TARGET IN ANOTHER DISEASE MODEL. SO, FOR EXAMPLE, IF YOU LOOKED AT CARDIAC ARRHYTHMIAS WHERE YOU HAVE A CLEAR, KNOWN PHENOTYPE AND IF YOU LOOKED AT DRUGS THAT ARE KNOWN TO ACT ON IT IN ORDER TO TEST THE TECHNOLOGY AND SHOW THE PROOF OF CONCEPT.

DR. PRIETO: TECHNICALLY WE DON'T REQUIRE THAT SORT OF PRELIMINARY DATA FOR THIS ROUND, CORRECT?

DR. SAMBRANO: WE DO NOT.

DR. PRIETO: OKAY. THANK YOU.

CHAIRMAN THOMAS: OTHER QUESTIONS?

DR. MARTIN: QUESTION. THERE WAS AN APPEAL LETTER. THAT I DON'T REMEMBER, BUT WAS THAT FOR THE SAME APP?

CHAIRMAN THOMAS: NO. THAT WAS FOR ONE OF THE TRANSLATIONAL APPLICATIONS.

MS. BONNEVILLE: THERE IS A LETTER FOR THIS APPLICATION THAT WAS SUBMITTED, J.T.

CHAIRMAN THOMAS: I'M SORRY. I THOUGHT YOU WERE REFERRING TO THE ONE IN THE TRANSLATIONAL.

MS. BONNEVILLE: THERE IS ONE THERE AS

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

WELL.

CHAIRMAN THOMAS: THANK YOU, MARIA. THANK YOU FOR CORRECTING ME ON THAT.

DR. JUELSGAARD: WHICH APPLICATION NUMBER ARE WE TALKING ABOUT AGAIN?

DR. SAMBRANO: THIS IS 10674.

DR. JUELSGAARD: OKAY. THANK YOU.

CHAIRMAN THOMAS: DR. SAMBRANO, DO WE HAVE A COMMENT ON THIS APPLICATION BY THE TEAM?

DR. SAMBRANO: WE DO NOT.

CHAIRMAN THOMAS: OKAY. I, FOR ONE, BASED ON THE EXCHANGE WITH DR. PRIETO AND DR. SAMBRANO THAT WE JUST HAD, WHICH REFERENCED THAT PARTICULAR CRITERIA THAT SEEMS TO HAVE BEEN THE MAJOR ISSUE HERE ISN'T ONE THAT TYPICALLY APPLIES FOR A INCEPTION AWARD. AND GIVEN THE NEED FOR GREAT, NEW IDEAS IN PARKINSON'S, I, FOR ONE, WOULD SUPPORT MOVING THIS APPLICATION FROM TIER II TO TIER I.

DR. HIGGINS: AS WOULD I.

MS. WINOKUR: SO WOULD I.

DR. MARTIN: IN THE CONTEXT OF THE APPEAL LETTER THAT, I THINK, ADDRESSED, AT LEAST IN MY MIND, WHAT THE ISSUES WERE, I WOULD ALSO. THIS IS DAVE MARTIN.

CHAIRMAN THOMAS: THANK YOU, DR. MARTIN.

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

THANK YOU, MS. WINOKUR.

OKAY. IF THERE AREN'T ANY OTHER COMMENTS HERE, SO --

DR. STEWARD: I WILL ALSO VOTE IN FAVOR OF THIS, BUT I WOULD SAY NOT ON THE BASIS OF THE APPEAL LETTER, BUT RATHER ON THE BASIS OF DAVID'S COMMENTS AND GIL'S WITH RESPECT TO THE CRITERIA. I AM CONCERNED ABOUT ANY RE-REVIEW IN TERMS OF TECHNICAL MERIT AND ALWAYS DEFER TO THE GRANTS WORKING GROUP FOR THEIR ORIGINAL SCORING. HOWEVER, I AM MOVED BY DAVID'S APPEAL ON THIS, AND I DO THINK THAT IT'S WORTH THIS AMOUNT OF MONEY TO GIVE THIS A TRY. THANK YOU.

CHAIRMAN THOMAS: THANK YOU, DR. STEWARD.

ANY OTHER COMMENTS FROM MEMBERS OF THE BOARD? SO TO MOVE THIS FROM TIER II TO TIER I, WE HAVE A MOTION. MARIA, WILL YOU PLEASE CALL THE ROLL.

MS. BONNEVILLE: WE WOULD NEED TO TAKE PUBLIC COMMENT FIRST.

CHAIRMAN THOMAS: SORRY. PUBLIC COMMENT? I THINK WE DO HAVE PUBLIC COMMENT HERE DOWN IN LA JOLLA.

DR. NIENABER: THANK YOU. THIS IS DR. NIENABER, PI ON THE GRANT. I HAVE WORKED IN

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

DRUG DISCOVERY FOR 30 YEARS, AND IT'S CLEAR TO ME NOW THAT WE NEED A PARADIGM SHIFT IN HOW WE APPROACH DIFFICULT DISEASES LIKE PARKINSON'S. WE CHOSE TO FOCUS ON PARKINSON'S DISEASE BECAUSE IT SPEAKS TO AN UNMET NEED, EVEN THOUGH WE KNEW IT WOULD BE MORE CHALLENGING THAN OTHER DISEASES.

AT ZENOBIA WE HAVE THERAPY FOR PD, OUR DISCOVERY PARADIGM THAT WORKED FOR CANCER AND OTHER DISEASES, BUT HAVE NOT WORKED FOR PD. LIKE THOSE BEFORE US, OUT COMPOUNDS BEHAVE VERY WELL IN MODELS, BUT SOME DO NOT BEHAVE AS PREDICTED IN NEURONS IN AN ANIMAL MODEL. TO OVERCOME THESE LIMITATIONS, WE DEVELOPED A NOVEL APPROACH WHICH LED US TO CIRM AND THE DISC1 GRANT.

WE'RE VERY EXCITED TO LEARN THAT THE REVIEWERS BELIEVE WE HAVE A, QUOTE, BY CHANCE OF IDENTIFYING A NOVEL CANDIDATE DRUG FOR PD, THAT WE UNDERSTAND AND ADDRESS MANY OF THE CHALLENGES IN THE FIELD, AND THAT OUR SCIENCE IS SOLID AND INNOVATIVE. OUR TECHNOLOGY IDENTIFIES COMPOUNDS THAT IMPROVE PARKINSON GENOTYPES DIRECTLY IN PATIENT-DERIVED NEURONS AND IDENTIFIES A UNIQUE SET OF CLINICAL REACTION. THIS COULD TAKE YEARS OFF THE DISCOVERY TIMELINE BY REMOVING THE NEED FOR EARLY ASSUMPTIONS AND MODEL SYSTEMS. OTHER SCREENING METHODS REQUIRE

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

BIASED STUDIES WHICH LEAD BACK TO THE SAME OLD ISSUE.

FURTHERMORE, PARKINSON'S IS LIKELY TO ADVANCE DISEASE, AND IT IS UNLIKELY THAT ONE SINGLE THERAPY WILL WORK FOR ALL PATIENTS. OUR GOAL IS TO IDENTIFY PERSONALIZED TREATMENTS AND SIDE-BY-SIDE DIAGNOSTIC TESTS FOR CLINICAL TRIALS IN PATIENT-DERIVED CELLS. AFTER PD THERE IS NO REASON WHY THIS TECHNOLOGY COULD NOT BE BROADENED TO OTHER RELATED DISEASES SUCH AS ALZHEIMER'S, HUNTINGTON'S, ALS, OR EVEN BRAIN INJURY.

AS YOU DISCUSSED, THE PRIMARY CONCERN OF THE REVIEWERS IS THAT WE DIDN'T PROVIDE PRELIMINARY DATA, AND THIS WAS A MISUNDERSTANDING IN READING THE RFA, BUT I UNDERSTAND IT'S HELPFUL. TO CLARIFY, THE CHEMISTRY HAS BEEN VALIDATED IN THE LABORATORY BY OUR COLLABORATOR, DR. BARRY SHARPLESS, WHO RECEIVED THE NOBEL PRIZE IN CHEMISTRY IN 2001. SPECIFICALLY, AS HE PUBLISHED LAST YEAR, "THE CHEMISTRY (INAUDIBLE) HAS BEEN IDENTIFIED AND VALIDATED AS TARGETS OF THE COMPOUND. IN HIS MIND THERE IS NO ISSUE TRANSLATING FROM HUMAN TO NEURONAL CELLS.

DR. JOHN MOSES (INAUDIBLE.)

THE REPORTER: I'M SORRY, MR. CHAIRMAN. THERE IS INTERFERENCE ON THE LINE AND I'M UNABLE TO

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

UNDERSTAND.

MR. TORRES: HOW ABOUT CALLING FOR THE QUESTION?

CHAIRMAN THOMAS: I THINK SENATOR TORRES CALLED FOR THE QUESTION, WHICH I THINK THE MEMBERS OF THE SUBCOMMITTEE UNDERSTAND AND ARE READY TO VOTE HERE. THANK YOU, MR. SENATOR. I'M NOT SURE WHAT THAT STATIC WAS, BUT IS THERE ANY OTHER PUBLIC COMMENT ON THIS? MARIA, WILL YOU PLEASE CALL THE ROLL.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

DR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: YES.

MS. BONNEVILLE: ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

MR. SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
CHAIRMAN THOMAS: OKAY. THANK YOU. MR. SUPERVISOR, CAN I TURN THIS OVER TO YOU AT THIS POINT? WE ARE AT THE STAGE ON AGENDA ITEM NO. 4 WHERE I WAS ABOUT TO ASK IF THERE ARE ANY MEMBERS OF THE SUBCOMMITTEE THAT WOULD LIKE TO MOVE ANY OF THE PROJECTS FROM TIER I DOWN TO TIER II.
SUPERVISOR SHEEHY: YOU CAN GO AHEAD, J.T. I'VE GOT A BIT OF CATCHING UP TO DO.
CHAIRMAN THOMAS: THANK YOU.
MS. BONNEVILLE: YOU MAY WANT TO CONFIRM THAT THE SUBCOMMITTEE DOESN'T HAVE ANY OTHER APPLICATIONS THAT THEY'D LIKE TO MOVE UP BEFORE WE MOVE ON.
CHAIRMAN THOMAS: THANK YOU, MARIA. ARE THERE ANY OTHER APPLICATIONS THAT ANY MEMBERS OF THE

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

SUBCOMMITTEE WOULD LIKE TO MOVE UP FROM TIER II TO TIER I? OKAY.

HEARING NONE, ARE THERE ANY OF THE APPLICATIONS ANY MEMBERS OF THE SUBCOMMITTEE WOULD LIKE TO MOVE DOWN FROM TIER I TO TIER II?

HEARING NONE, I WOULD LIKE TO ENTERTAIN A MOTION THAT WE APPROVE ALL APPLICATIONS IN TIER I, INCLUDING THAT THAT WE JUST MOVED UP, AND NOT TO APPROVE THOSE APPLICATIONS IN TIER II. DO I HEAR SUCH A MOTION?

DR. HIGGINS: SO MOVED.

CHAIRMAN THOMAS: MOVED BY DR. HIGGINS, SECONDED BY?

DR. PRIETO: SECOND.

CHAIRMAN THOMAS: SECONDED BY DR. PRIETO. SO WE ARE MOVED AND APPROVED. DO WE HAVE ANY PUBLIC COMMENT ON THIS MOTION? HEARING NONE, MARIA, WILL YOU PLEASE CALL THE ROLL.

MR. TOCHER: AGAIN, I'D JUST LIKE TO INSTRUCT THE BOARD MEMBERS TO VOTE AYE OR NAY EXCEPT FOR THOSE WITH WHICH THEY HAVE A CONFLICT. I CAN SIMPLIFY IT. OS, IT'S JUST YOU TODAY.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

DR. JUELSGAARD: YES.
MS. BONNEVILLE: DAVE MARTIN.
DR. MARTIN: YES.
MS. BONNEVILLE: LAUREN MILLER.
MS. MILLER: YES.
MS. BONNEVILLE: ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: YES, EXCEPT FOR THOSE WITH WHICH I HAVE A CONFLICT.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
CHAIRMAN THOMAS: THANK YOU, MARIA. THAT CONCLUDES ITEM NO. 4.

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

ARE THERE ANY PUBLIC COMMENTS ON ANY TOPICS THAT MEMBERS OF THE PUBLIC WOULD LIKE TO SPEAK ON AT THIS POINT? HEARING NONE, THAT CONCLUDES TODAY'S MEETING. WE STAND ADJOURNED. THANK YOU VERY MUCH, EVERYBODY.

(THE MEETING WAS THEN CONCLUDED AT 11:36 A.M.)

133 HENNA COURT, SANDPOINT, IDAHO 83864
208-255-5453 208-920-3543 DRAIBE@HOTMAIL.COM

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 NOVEMBER 30, 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.

BETH C. DRAIN, CA CSR 7152
133 HENNA COURT
SANDPOINT, IDAHO
(208) 255-5453