

BETH C. DRAIN, CA CSR NO. 7152

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2
3 BEFORE THE
4 APPLICATION REVIEW SUBCOMMITTEE OF THE
5 INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE
6
7 TO THE
8 CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
9 ORGANIZED PURSUANT TO THE
10 CALIFORNIA STEM CELL RESEARCH AND CURES ACT
11
12 REGULAR MEETING

13 LOCATION: VIA ZOOM

14
15 DATE: APRIL 24, 2025
16 9 A.M.

17
18 REPORTER: BETH C. DRAIN, CA CSR
19 CSR. NO. 7152

20
21 FILE NO.: 2025-11
22
23
24
25

I N D E X

ITEM DESCRIPTION	PAGE NO.
OPEN SESSION	
1. CALL TO ORDER	2
2. ROLL CALL	2
3. CONSIDERATION OF AN APPLICATION SUBMITTED IN RESPONSE TO CLINICAL TRIAL STAGE PROJECTS PROGRAM ANNOUNCEMENTS (CLIN 1, 2, OR 4)	4
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 APPLICATIONS SUBMITTED IN RESPONSE TO AGENDA ITEM 3 ABOVE. (HEALTH & SAFETY CODE 125290.30(F) (3) (B) AND (C)).	
OPEN SESSION	
5. GENERAL COMMENTS ON ARS PROCESS	NONE
6. PUBLIC COMMENT	NONE
7. ADJOURNMENT	12

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APRIL 24, 2025; 9 A.M.

VICE CHAIR BONNEVILLE: I'D LIKE TO CALL
THE MEETING OF THE APPLICATION REVIEW SUBCOMMITTEE
TO ORDER. SCOTT, CAN YOU PLEASE TAKE THE ROLL.

MR. TOCHER: MARIA BONNEVILLE.

VICE CHAIR BONNEVILLE: PRESENT.

MR. TOCHER: JUDY CHOU. LEONDR
CLARK-HARVEY.

DR. CLARK-HARVEY: PRESENT.

MR. TOCHER: ANNE-MARIE DULIEGE.
YSABEL DURON.

MS. DURON: HERE.

MR. TOCHER: MARK FISCHER-COLBRIE.

MR. FISCHER-COLBRIE: HERE.

MR. TOCHER: ELENA FLOWERS. DAVID
HIGGINS.

DR. HIGGINS: PRESENT.

MR. TOCHER: VITO IMBASCIANI. RICH
LAJARA.

MR. LAJARA: PRESENT.

MR. TOCHER: CHRIS MIASKOWSKI.

DR. MIASKOWSKI: PRESENT.

MR. TOCHER: ADRIANA PADILLA.

DR. PADILLA: HERE.

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1 MR. TOCHER: JOE PANETTA.
2 MR. PANETTA: HERE.
3 MR. TOCHER: MARV SOUTHARD.
4 DR. SOUTHARD: HERE.
5 MR. TOCHER: KAROL WATSON.
6 DR. CHOU: JUDY CHOU IS HERE.
7 MR. TOCHER: THANK YOU, JUDY.
8 Yael. KEVIN XU.
9 DR. XU: HERE.
10 MR. TOCHER: Yael. THERE'S Yael. A WAVE.
11 OKAY. THAT'S GOOD ENOUGH FOR THE RECORD. WE'RE
12 GOOD TO GO, MARIA.
13 VICE CHAIR BONNEVILLE: THANK YOU.
14 DR. HAYLEY LAM WILL BE PRESENTING. SO, HAYLEY, AS
15 SOON AS YOU'RE READY, FEEL FREE.
16 DR. LAM: CAN EVERYONE SEE MY SCREEN?
17 GREAT. GOOD MORNING. I'M HERE TO PRESENT THE
18 SINGLE CLINICAL APPLICATION FOR YOUR CONSIDERATION
19 THIS MORNING.
20 AS ALWAYS, WE BEGIN WITH OUR MISSION,
21 ACCELERATING WORLD-CLASS SCIENCE TO DELIVER
22 TRANSFORMATIVE REGENERATIVE MEDICINE TREATMENTS IN
23 AN EQUITABLE MANNER TO A DIVERSE CALIFORNIA AND
24 WORLD.
25 THE STATE OF THE CURRENT CLINICAL BUDGET

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1 IS AS FOLLOWS: THERE WAS A BUDGET ALLOCATION
2 APPROVED BY THE BOARD IN DECEMBER FOR 76.7 MILLION
3 FOR THE LATTER HALF OF THE FISCAL YEAR. 50.4
4 MILLION HAS BEEN APPROVED THUS FAR BY THIS GROUP.
5 UP FOR CONSIDERATION TODAY IS 8 MILLION WHICH, IF
6 APPROVED, WOULD LEAVE A BALANCE OF A LITTLE BIT OVER
7 18 MILLION FOR THE REST OF THE FISCAL YEAR.

8 TO GIVE A REMINDER ON CLINICAL PROGRAM
9 PROCESS, THE SCORING SYSTEM FOR THE SCIENTIFIC
10 SCORING IS A 1, 2, AND 3. A 1 IS A RECOMMENDATION
11 FOR FUNDING FROM THE GRANTS WORKING GROUP. A TWO IS
12 A DO NOT RECOMMEND AT THIS TIME, BUT INVITES THE
13 APPLICATION TO RESUBMIT WITH REVISIONS BASED ON
14 FEEDBACK FROM THE GROUP. A SCORE OF 3 IS A DO NOT
15 FUND AT THIS TIME AND THE APPLICANT CANNOT RESUBMIT
16 THE SAME PROJECT FOR AT LEAST SIX MONTHS.

17 AND THE SCORING IS BASED ON THE FOLLOWING
18 FIVE REVIEW CRITERIA. DOES THE PROJECT UPHOLD THE
19 NECESSARY SIGNIFICANCE AND POTENTIAL FOR IMPACT?
20 WHAT'S THE OVERALL VALUE PROPOSITION OF THE PROJECT?
21 IS THE RATIONALE SOUND? DOES THE DATA SUPPORT
22 MOVING FORWARD? IS THE PROJECT WELL PLANNED AND
23 DESIGNED? SO ARE THE ACTIVITIES THAT THEY PROPOSED
24 FOR THE AWARD APPROPRIATE FOR WHAT THEY PLAN TO DO?
25 IS THE PROJECT FEASIBLE? DO THEY HAVE PEOPLE, TEAM,

1 RESOURCES IN PLACE TO EXECUTE? AND DOES THE PROJECT
2 UPHOLD THE PRINCIPLES OF DIVERSITY, EQUITY, AND
3 INCLUSION? SO WILL THEY CONSIDER PATIENT DIVERSITY
4 AND EQUITABLE ENROLLMENT?

5 THE CLINICAL PROGRAM ALSO HAS A DIVERSITY,
6 EQUITY, AND INCLUSION SCORING SYSTEM ON A SCALE OF
7 ZERO TO TEN, A 10 BEING AN OUTSTANDING RESPONSE.
8 AND THESE ARE SCORED BY THE GRANTS WORKING GROUP
9 BOARD MEMBERS WITH NO CONFLICT.

10 SO AS AN OVERVIEW, THE PANEL CONSISTS OF
11 THREE TYPES OF MEMBERS: THE SCIENTIFIC GRANTS
12 WORKING GROUP WHO PROVIDE THE SCIENTIFIC EVALUATION,
13 GRANTS WORKING GROUP BOARD MEMBERS WHO PROVIDE DEI
14 EVALUATION AND SUGGESTED SCIENTIFIC SCORES, AS WELL
15 AS OUR AD HOC SPECIALISTS WHO FILL IN WITH EXPERTISE
16 AS NEEDED ON THE PANEL.

17 SO WITHOUT FURTHER ADO, I WILL TRANSITION
18 INTO DISCUSSION OF THE SPECIFIC APPLICATION FOR YOUR
19 CONSIDERATION TODAY, WHICH IS CLIN2-17086,
20 OPTOGENETIC GENE THERAPY FOR THE TREATMENT OF
21 RETINITIS PIGMENTOSA.

22 THE APPLICANT IS REQUESTING 7.975 MILLION
23 WITH REQUIRED 30 PERCENT CO-FUNDING OF 3.4 MILLION.
24 AND THIS IS A CALIFORNIA ORGANIZATION SEEKING
25 FUNDING TO COMPLETE A PHASE 1 TRIAL.

1 A LITTLE BIT OF BACKGROUND ABOUT THIS
2 PROJECT. RETINITIS PIGMENTOSA IS AN INHERITED
3 GENETIC DISEASE WHERE THERE'S ESSENTIALLY
4 PROGRESSIVE LOSS OF VISION OVER TIME. THE PATIENTS
5 ARE TYPICALLY DIAGNOSED IN LATE TEENS, EARLY
6 TWENTIES AND ON AVERAGE BY ABOUT 40 YEARS OF AGE ARE
7 LEGALLY BLIND. AND SO, OF COURSE, THIS WILL IMPACT
8 DAILY ACTIVITIES SUCH DRIVING AND ABILITY TO WORK.

9 THE CURRENT TREATMENT OPTIONS ARE VERY
10 LIMITED. AND RIGHT NOW THERE'S A GENE THERAPY FOR A
11 VERY SMALL SUBSET OF PEOPLE WITH A VERY SPECIFIC
12 GENE MUTATION. SO ONE OF THE VALUES OF THIS
13 PARTICULAR PRODUCT IS THAT IT'S GENE MUTATION
14 AGNOSTIC. THERE'S ABOUT A HUNDRED DIFFERENT
15 POSSIBLE MUTATIONS THAT COULD CAUSE RETINITIS
16 PIGMENTOSA. AND THE PROPOSED PROJECT COULD,
17 THEREFORE, TARGET A BROADER PATIENT POPULATION.

18 AND THE WAY THAT THIS PRODUCT WORKS IS
19 THAT IT DELIVERS A LIGHT-SENSITIVE GENE TO THE
20 REMAINING RETINAL CELLS AND ESSENTIALLY ALLOWS THEM
21 TO DETECT LIGHT.

22 AND THIS IS A CIRM PROJECT AS IT'S A GENE
23 THERAPY PRODUCT.

24 IN TERMS OF CIRM PORTFOLIO PROJECTS, THERE
25 IS ONE CURRENT, ACTIVE CLINICAL TRIAL PHASE AWARD

1 AND FOR THE SAME INDICATION, RETINITIS PIGMENTOSA.
2 THE CANDIDATE IS A STEM CELL PRODUCT AND IS INJECTED
3 SUBRETINALLY INTO THE EYE. AND THE MECHANISM OF
4 THIS PRODUCT IS DIFFERENT THAN THE CURRENT
5 APPLICATION UNDER CONSIDERATION. IT MODULATES
6 INFLAMMATION.

7 THE APPLICANT TEAM HAS RECEIVED PRIOR CIRM
8 FUNDING IN THE FORM OF TWO TRANSLATIONAL STAGE
9 AWARDS. ONE IS A DIRECT PROGRESSION OF THIS PROJECT
10 AT THE PRECLINICAL STAGE, AND THE OTHER IS AN ACTIVE
11 TRANSLATIONAL AWARD FOR A SIMILAR APPROACH FOR A
12 DIFFERENT INDICATION, GEOGRAPHIC ATROPHY, WHICH IS
13 AN AGE-RELATED RETINAL DEGENERATION INDICATION.

14 SO FINALLY, FOR YOUR CONSIDERATION,
15 CLIN2-17086, OPTOGENETIC GENE THERAPY FOR THE
16 TREATMENT OF RETINITIS PIGMENTOSA. THIS APPLICATION
17 RECEIVED A UNANIMOUS RECOMMENDATION FOR FUNDING FROM
18 THE GRANTS WORKING GROUP WITH A DEI SCORE OF 7, AND
19 THE CIRM TEAM CONCURS WITH THE RECOMMENDATION FROM
20 THE GRANTS WORKING GROUP TO FUND THE APPLICATION FOR
21 JUST UNDER 8 MILLION.

22 SO I'LL TURN IT BACK TO YOU, VICE CHAIR
23 BONNEVILLE.

24 VICE CHAIR BONNEVILLE: THANK YOU, HAYLEY.
25 DO I HAVE A MOTION TO ACCEPT THE TEAM'S

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1 RECOMMENDATION TO FUND APPLICATION NO. 17086?

2 DR. DULIEGE: MOVE.

3 VICE CHAIR BONNEVILLE: THAT'S ANNE-MARIE.

4 DR. SOUTHARD: I WILL SECOND.

5 VICE CHAIR BONNEVILLE: IT WAS A TIE
6 BETWEEN LEONDRA AND MARV.

7 DR. CLARK-HARVEY: I'LL GIVE IT TO MARV.

8 VICE CHAIR BONNEVILLE: THANK YOU. I WANT
9 TO OPEN IT UP TO BOARD COMMENT. DOES ANYONE ON THE
10 BOARD HAVE ANY COMMENTS OR QUESTIONS FOR THE TEAM?
11 OKAY. SEEING NONE, DO WE HAVE ANY PUBLIC COMMENT?

12 DR. BRESGE: I JUST WANT TO SAY I'M PAUL
13 BRESGE. I'M ONE OF THE CO-FOUNDERS OF RAY
14 THERAPEUTICS. I WANT TO THANK CIRM AND THE BOARD
15 VERY MUCH FOR SUPPORTING OUR VERY IMPORTANT WORK FOR
16 A VERY HIGH UNMET NEED. JUST LOTS OF GRATITUDE.
17 THANK YOU.

18 VICE CHAIR BONNEVILLE: THANK YOU, PAUL.
19 CLAUDE, SCOTT, ANY OTHER PUBLIC COMMENT?

20 MR. TOCHER: DOESN'T APPEAR SO.

21 VICE CHAIR BONNEVILLE: THANK YOU. WITH
22 THAT, WE CAN PROCEED TO A VOTE. SCOTT, CAN YOU
23 PLEASE CALL THE ROLL.

24 MR. TOCHER: SURE. THE MOTION IS TO FUND
25 THE APPLICATION 17086.

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1 DAN BERNAL. MARIA BONNEVILLE.
2 VICE CHAIR BONNEVILLE: YES.
3 MR. TOCHER: JUDY CHOU.
4 DR. CHOU: YES.
5 MR. TOCHER: LEONDRA CLARK-HARVEY.
6 DR. CLARK-HARVEY: YES.
7 MR. TOCHER: ANNE-MARIE DULIEGE.
8 DR. DULIEGE: YES.
9 MR. TOCHER: YSABEL DURON.
10 MS. DURON: YES.
11 MR. TOCHER: MARK FISCHER-COLBRIE.
12 MR. FISCHER-COLBRIE: YES.
13 MR. TOCHER: ELENA FLOWERS. DAVID
14 HIGGINS.
15 DR. HIGGINS: YES.
16 MR. TOCHER: VITO IMBASCIANI. RICH
17 LAJARA.
18 MR. LAJARA: YES.
19 MR. TOCHER: CHRIS MIASKOWSKI.
20 DR. MIASKOWSKI: YES.
21 MR. TOCHER: ADRIANA PADILLA.
22 DR. PADILLA: YES.
23 MR. TOCHER: JOE PANETTA.
24 MR. PANETTA: YES.
25 MR. TOCHER: MARV SOUTHARD.

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1 DR. SOUTHARD: YES.

2 MR. TOCHER: KAROL WATSON.

3 DR. WATSON: YES.

4 MR. TOCHER: Yael WYTE.

5 DR. WYTE: YES.

6 MR. TOCHER: KEVIN XU.

7 DR. XU: YES.

8 MR. TOCHER: THANK YOU VERY MUCH. MADAM
9 VICE CHAIR, THE MOTION CARRIES.

10 VICE CHAIR BONNEVILLE: THANK YOU. NEXT,
11 ARE THERE ANY GENERAL QUESTIONS ON THE APPLICATION
12 REVIEW SUBCOMMITTEE PROCESS? IF THERE ARE NONE,
13 THIS MEETING IS ADJOURNED.

14 DR. DULIEGE: IT'S NOT A QUESTION ABOUT
15 THE PROCESS OR SO, BUT I WANTED TO SAY HOW THAT'S AN
16 EXAMPLE OF HOW GREAT CIRM WORKS. WE ARE PROVIDING
17 SUPPORT FOR THE TRANSLATIONAL STAGE OF THIS
18 APPLICATION NOW FOR PHASE 1, HOPEFULLY FOR MORE DOWN
19 THE ROAD, IN AN EXTRAORDINARILY IMPORTANT
20 INDICATION, YET WE HAVE INVESTED IN VARIOUS MODES OF
21 DELIVERY OF CELL AND GENE THERAPY THAT'S CIRM AT ITS
22 BEST.

23 VICE CHAIR BONNEVILLE: THANK YOU,
24 ANNE-MARIE. I AGREE. SO THANK YOU FOR SAYING THAT.
25 IF THERE ARE NO OTHER FURTHER COMMENTS OR

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1 ANY OTHER PUBLIC COMMENTS, I THINK WE ARE DONE AND
2 OUR BUSINESS IS DONE. SO THANK YOU ALL SO MUCH FOR
3 JOINING US, FOR ME IT'S AN AFTERNOON, BUT FOR YOU
4 THIS MORNING, AND WE'LL SEE YOU SOON.

5 (THE MEETING WAS THEN CONCLUDED AT 9:13 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 VIRTUAL PROCEEDINGS BEFORE THE APPLICATION REVIEW SUBCOMMITTEE OF THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON APRIL 24, 2025, 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) 920-3543