INDEPENDE CALIFORNIA I O	BEFORE THE TION REVIEW SUBCOMMITTEE OF THE NT CITIZENS' OVERSIGHT COMMITTEE TO THE INSTITUTE FOR REGENERATIVE MEDICINE RGANIZED PURSUANT TO THE STEM CELL RESEARCH AND CURES ACT REGULAR MEETING
LOCATION:	VIA ZOOM
DATE:	MAY 29, 2025 9 A.M.
REPORTER:	BETH C. DRAIN, CA CSR CSR. NO. 7152
FILE NO.:	2025-13

		BETH C. DRAIN, CA CSR NO. 7152	
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2		INDEX	
3			
4	ITE	M DESCRIPTION	PAGE NO.
5	OPE	N SESSION	
6	1.	CALL TO ORDER	3
7	2.	ROLL CALL	3
8 9	3. CONSIDERATION OF AN APPLICATION 4 SUBMITTED IN RESPONSE TO CLINICAL TRIAL STAGE PROJECTS PROGRAM ANNOUNCEMENTS	4	
10		IN 1, 2, OR 4)	
11		SED SESSION	NONE
12	4.		
13	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)).		
14 15			
16	OPE	N SESSION	
17	5.	GENERAL COMMENTS ON ARS PROCESS	NONE
18	6.	PUBLIC COMMENT	NONE
19	7.	ADJOURNMENT	15
20			
21			
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23			
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25			
		2	

1	MAY 29, 2025; 9 A.M.
2	
3	CHAIRMAN IMBASCIANI: OKAY. GOING TO CALL
4	TO ORDER THIS MEETING OF THE APPLICATION REVIEW
5	SUBCOMMITTEE. THANK YOU ALL FOR ATTENDING ON THIS
6	BEAUTIFUL THURSDAY MORNING. SCOTT, WE CAN START
7	WITH A ROLL CALL.
8	MR. TOCHER: CERTAINLY. DAN BERNAL.
9	MR. BERNAL: PRESENT.
10	MR. TOCHER: MARIA BONNEVILLE.
11	VICE CHAIR BONNEVILLE: PRESENT.
12	MR. TOCHER: JUDY CHOU. LEONDRA
13	CLARK-HARVEY.
14	DR. CLARK-HARVEY: PRESENT.
15	MR. TOCHER: ANNE-MARIE DULIEGE. YSABEL
16	DURON.
17	MS. DURON: HERE.
18	MR. TOCHER: MARK FISCHER-COLBRIE.
19	DR. FISCHER-COLBRIE: HERE.
20	MR. TOCHER: ELENA FLOWERS.
21	DR. FLOWERS: PRESENT.
22	MR. TOCHER: DAVID HIGGINS.
23	DR. HIGGINS: HERE.
24	MR. TOCHER: VITO IMBASCIANI.
25	CHAIRMAN IMBASCIANI: HERE.
	3

1	MR. TOCHER: RICH LAJARA.
2	MR. LAJARA: PRESENT.
3	MR. TOCHER: CHRISTINE MIASKOWSKI.
4	DR. MIASKOWSKI: PRESENT.
5	MR. TOCHER: ADRIANA PADILLA.
6	DR. PADILLA: HERE.
7	MR. TOCHER: JOE PANETTA.
8	MR. PANETTA: HERE.
9	MR. TOCHER: MARVIN SOUTHARD.
10	DR. SOUTHARD: HERE.
11	MR. TOCHER: KAROL WATSON. YAEL WYTE.
12	DR. WYTE: HERE.
13	MR. TOCHER: KEVIN XU.
14	DR. DULIEGE: SCOTT, THIS IS ANNE-MARIE
15	AND I'M HERE.
16	MR. TOCHER: GREAT. THANK YOU,
17	ANNE-MARIE. EXCELLENT. WE HAVE A QUORUM OF 15
18	MEMBERS.
19	CHAIRMAN-IMBASCIANI: BEAUTIFUL. THANK
20	YOU VERY MUCH. WE'RE GOING TO CONSIDER AN
21	APPLICATION SUBMITTED IN RESPONSE TO OUR CLINICAL
22	TRIAL STAGE PROJECTS PROGRAM ANNOUNCEMENT. AND I AM
23	GOING TO PASS THE MICROPHONE, SO TO SPEAK, OVER TO
24	DR. HAYLEY LAM FOR THE PRESENTATION. THANK YOU,
25	HAYLEY.

4

1	DR. LAM: THANK YOU, CHAIR IMBASCIANI.
2	GIVE ME A MOMENT HERE TO SHARE MY SLIDES. ALL
3	RIGHT. CAN FOLKS SEE THAT? THANK YOU.
4	SO MY PLEASURE THIS MORNING TO PRESENT THE
5	GRANTS WORKING GROUP RECOMMENDATIONS FOR THE
6	CLINICAL PROGRAM. AS ALWAYS, WE BEGIN WITH OUR
7	MISSION: ACCELERATING WORLD-CLASS SCIENCE TO
8	DELIVER TRANSFORMATIVE REGENERATIVE MEDICINE
9	TREATMENTS IN AN EQUITABLE MANNER TO A DIVERSE
10	CALIFORNIA AND WORLD.
11	THE CURRENT CLINICAL BUDGET STATUS IS AS
12	FOLLOWS. THERE WAS 76.7 MILLION APPROVED BY THE
13	BOARD FOR THE REMAINDER OF THE FISCAL YEAR. AS OF
14	RIGHT NOW, 58.4 HAS BEEN APPROVED BY THIS GROUP.
15	AND TODAY WE HAVE 4.7 MILLION REQUESTED, AND THAT
16	WOULD LEAVE ABOUT JUST UNDER 14 MILLION REMAINING IF
17	APPROVED.
18	THE CLINICAL SCORING SYSTEM IS A SYSTEM OF
19	1-2-3. AND 1 IS A RECOMMENDATION FOR FUNDING. A 2
20	IS A NOT FUND AT THIS TIME, BUT INVITES THE
21	APPLICANT TO RESUBMIT WITH FIXABLE CHANGES. AND $3$
22	IS DO NOT RECOMMEND FOR FUNDING AND THE PROJECT
23	CANNOT RETURN FOR A FEW MONTHS.
24	THE SCIENTIFIC MEMBERS OF THE WORKING
25	GROUP SCORE HOLISTICALLY A 1-2-3 BASED ON THE
	5

1	SCIENTIFIC REVIEW CRITERIA WHICH ARE AS FOLLOWS. SO
2	DOES THE PROJECT HOLD THE NECESSARY SIGNIFICANCE AND
3	POTENTIAL IMPACT? SO DOES IT HAVE OVERALL VALUE?
4	IS IT WORTH PROCEEDING? SECONDLY, IS THE RATIONALE
5	SOUND? SO DOES THE BODY OF DATA THAT THEY HAVE
6	GATHERED SUPPORT MOVING FORWARD? IS THE PROJECT
7	WELL-PLANNED AND DESIGNED? SO ARE THE ACTIVITIES
8	AND THINGS THAT THEY PLAN TO DO WITH CIRM FUNDING
9	APPROPRIATE FOR THE GOAL OF THE PROJECT? AND IS THE
10	PROJECT FEASIBLE? SO DO THEY HAVE THE PEOPLE, TEAM,
11	LEADERSHIP, AND CONTRACTORS, AND ALL THE RESOURCES
12	IN PLACE TO EXECUTE THE PROJECT IN A TIMELY WAY?
13	THE REVIEW PANEL IS COMPOSED OF THREE
14	DIFFERENT TYPES OF FOLKS. SO WE HAVE UP TO 15
15	SCIENTIFIC GRANTS WORKING GROUP MEMBERS THAT PROVIDE
16	A SCIENTIFIC EVALUATION OF THE DISEASE AREA,
17	REGULATORY, MANUFACTURING, PRODUCT DEVELOPMENT.
18	THEY PROVIDE A SCIENTIFIC SCORE ON ALL THE
19	APPLICATIONS. WE HAVE OUR GRANTS WORKING GROUP
20	BOARD MEMBERS WHO SUGGEST A SCIENTIFIC SCORE AND
21	PROVIDE A PATIENT PERSPECTIVE ON THE POTENTIAL
22	IMPACT OF THE PROJECT. AND WE ALSO HAVE OUR AD HOC
23	SPECIALISTS THAT PROVIDE EXPERTISE THAT MAY NOT BE
24	COVERED BY THE STANDING PANEL, AND THEY PROVIDE
25	INITIAL, BUT NOT A FINAL SCIENTIFIC SCORE.

6

1	SO WITH THAT, I WILL THIS DOESN'T
2	INCLUDE SORRY. THERE IS ONE CONFLICT ON THE
3	GROUP TODAY, BUT I THINK YOU KNOW WHO YOU ARE.
4	SO ON STRAIGHT TO THE APPLICATION. SO FOR
5	YOUR CONSIDERATION TODAY IS CLIN1-17090, DEVELOPMENT
6	OF A NOVEL INTRAVITREAL AAV GENE THERAPY FOR THE
7	RARE DISEASE BLUE CONE MONOCHROMACY. THIS IS A GENE
8	THERAPY FOR THE INDICATION. AND THE APPLICANT IS
9	SEEKING FUNDING FOR IND APPROVAL IN THE AMOUNT OF
10	4.691 MILLION. AND THE APPLICANT DOES NOT HAVE
11	REQUIRED CO-FUNDING AND IS A CALIFORNIA
12	ORGANIZATION.
13	A LITTLE BIT OF BACKGROUND ABOUT THE
14	PROJECT. SO THIS IS A RARE, INHERITED DISEASE
15	ESSENTIALLY WHERE THERE ARE MUTATIONS IN THE
16	FUNCTION IN THE RED AND GREEN PHOTORECEPTORS IN THE
17	RETINA. AND SO THIS IS USUALLY DIAGNOSED IN INFANCY
18	AND IMPACTS THE QUALITY OF LIFE OF PEOPLE WITH THIS
19	CONDITION CAUSING LOW VISION, PHOTOSENSITIVITY. SO
20	SENSITIVITY TO LIGHT AND IMPAIRED COLOR
21	DISCRIMINATION AND, OF COURSE, ALSO SOME
22	UNCONTROLLED EYE MOVEMENT IS POSSIBLE AS WELL.
23	THE CURRENT TREATMENTS ARE SYMPTOMATIC
24	ONLY. SO SORT OF VISION AIDS AND SUNGLASSES, THAT
25	SORT OF THING. THE PROPOSED THERAPY IS DESIGNED AS
	7

1	A ONE-TIME INJECTION INTO THE EYE AND AIMS TO
2	ESSENTIALLY EXPRESS A FUNCTIONAL COPY OF THE L-OPSIN
3	GENE IN THE PHOTORECEPTORS AND HOPEFULLY RESTORE
4	VISUAL FUNCTION. SO THIS WOULD BE A FUNCTIONAL
5	IMPROVEMENT ESSENTIALLY FOR THE PATIENTS THAT HAVE
6	THIS DISEASE AS OPPOSED TO MANAGING SYMPTOMS ONLY.
7	AND THE RELEVANCE TO CIRM IS THIS IS A GENE THERAPY
8	PRODUCT.
9	CIRM DOES NOT CURRENTLY HAVE ANY
10	TRANSLATIONAL OR CLINICAL AWARDS ADDRESSING THIS
11	INDICATION. AND THE APPLICANT HAS NOT RECEIVED
12	PRIOR CIRM FUNDING.
13	SO IN SUMMARY FOR CONSIDERATION TODAY IS
14	CLIN1-17090. THIS APPLICATION RECEIVED A UNANIMOUS
15	RECOMMENDATION FOR FUNDING WITH 15 VOTES TO A SCORE
16	OF 1 FROM THE SCIENTIFIC MEMBERS OF THE GRANTS
17	WORKING GROUP, AND THE CIRM TEAM CONCURS WITH THIS
18	RECOMMENDATION TO FUND THE APPLICATION FOR THE
19	AMOUNT LISTED HERE, JUST UNDER 4.7 MILLION.
20	AND I WILL HAND IT BACK TO CHAIR
21	IMBASCIANI FOR DISCUSSION.
22	CHAIRMAN IMBASCIANI: THANK YOU, HAYLEY.
23	I'M GOING TO OPEN THE DISCUSSION ON THIS
24	GRANT APPLICATION TO BOARD MEMBERS. STARTING OFF
25	THE DISCUSSION, I'M VERY HAPPY. I KNOW OUR
	8

1	PORTFOLIO IS VERY RICH WITH OTHER DISEASES THAT
2	AFFECT THE RETINA OR THE CORNEA, OTHER PARTS OF THE
3	EYE. HAYLEY MENTIONS THAT THIS IS THE FIRST TIME
4	THAT WE HAVE CONSIDERED FUNDING SOMETHING THAT LOOKS
5	AT RODS AND CONES THAT CAUSE VISUAL IMPAIRMENT. SO
6	I'M VERY HAPPY TO SEE THAT, EVEN THOUGH OUR
7	PORTFOLIO IS RICH IN EYE-RELATED DISEASES, THAT WE
8	CONTINUE TO EXPAND THE UNMET NEED.
9	SO ANY OTHER BOARD MEMBERS WANT TO MAKE
10	COMMENTS ON THE APPLICATION? CAN WE START WITH A
11	MOTION? THE CHAIR WILL ENTERTAIN TO ACCEPT THE
12	RECOMMENDATION OF THE WORKING GROUP.
13	DR. SOUTHARD: SO MOVED.
14	CHAIRMAN IMBASCIANI: MARVIN. THANK YOU.
15	MS. DURON: SECOND.
16	MR. TOCHER: AND THE SECOND IS BY YSABEL
17	DURON.
18	CHAIRMAN IMBASCIANI: THANK YOU, YSABEL.
19	OKAY. FLOOR IS OPEN TO DISCUSSION. JOE PANETTA.
20	MR. PANETTA: THANKS, VITO. I CAN'T FIND
21	MY LITTLE HAND RAISE HERE.
22	HAYLEY, THANKS AS USUAL FOR A FANTASTIC
23	PRESENTATION.
24	JUST A COMMENT FIRST OF ALL, THAT THIS IS
25	ONE OF THOSE THINGS THAT I BELIEVE IS A THERAPY THAT
	9

1	IS A PERFECT OPPORTUNITY, HOPEFULLY, FOR CIRM TO
2	REALLY SHOW ITS VALUE IF THIS WORKS. IT'S A GENE
3	THERAPY. I KNOW WE'VE GOT A LOT OF VISION-RELATED
4	THERAPIES IN DEVELOPMENT, BUT THIS IS ONE OF THOSE
5	THINGS THAT STRIKES ME THAT IF THIS WORKS, IT'S A
6	REAL OPPORTUNITY TO SAY WE MADE AN IMPACT.
7	I'M ASSUMING THE PREVALENCE OF THIS IN THE
8	POPULATION IS NOT GREAT, BUT THAT WAS MY OTHER
9	QUESTION.
10	DR. LAM: PREVALENCE WORLDWIDE IS ONE IN A
11	100,000. AND THERE IS ESTIMATED ABOUT 3,000 FOLKS
12	IDENTIFIED WITH THIS IN THE UNITED STATES.
13	MR. PANETTA: THANKS.
14	CHAIRMAN IMBASCIANI: NO ONE WANTS TO
15	SPARE THE ROD TO SAVE THE CONE. WE CAN NO
16	FURTHER DISCUSSION FROM BOARD MEMBERS.
17	DR. LAM: THERE IS A HAND RAISED ON THE
18	PHONE.
19	MR. TOCHER: IT'S FOR PUBLIC COMMENT.
20	MS. MANDAC: THEY'RE PUBLIC COMMENT.
21	CHAIRMAN IMBASCIANI: I'M GOING TO SEGUE
22	TO PUBLIC COMMENT NOW. WILL THE INDIVIDUAL WHO'S
23	CALLING IN PLEASE IDENTIFY THEMSELVES. AND YOU HAVE
24	THREE MINUTES TO ADDRESS THE BOARD. THANK YOU.
25	MS. MANDAC: BEFORE YOU START, THERE IS
	10

1	GOING TO BE A TIMER. WE WILL MUTE YOU WHEN YOUR
2	TIME STARTS. WE'LL START WITH THE NUMBER ENDING IN
3	9994. YOUR TIME BEGINS NOW. PHONE NUMBER
4	281-302-9994, YOU HAVE THE FLOOR. YOU'LL NEED TO
5	UNMUTE.
6	MR. CAVITT: HELLO. MY NAME IS JOHN
7	CAVITT, AND I AM THE CO-FOUNDER OF BLUEGEN
8	THERAPEUTICS FOUNDATION AND FATHER OF THREE CHILDREN
9	WITH BCM. SO ON BEHALF OF OUR TEAM, THE MANY
10	SCIENTISTS WHO'VE WORKED TO GET HERE, AND THE
11	COUNTLESS FAMILIES THAT WILL BE IMPACTED BY THIS
12	POTENTIAL THERAPY. I WANT TO THANK YOU FOR ALL THE
13	DILIGENCE THAT WAS DONE ON THIS PROGRAM. SO THANK
14	YOU.
15	CHAIRMAN IMBASCIANI: IS THERE ANY OTHER
16	MEMBER OF THE PUBLIC WHO WANTS TO SPEAK?
17	MS. MANDAC: YES. WE DO HAVE TWO OTHER
18	MEMBERS OF THE PUBLIC WITH THEIR HANDS RAISED. THE
19	NEXT PHONE NUMBER WILL BE THE 212-920-9834. YOU
20	HAVE THE FLOOR. IF YOU COULD PLEASE UNMUTE.
21	DR. NAGIEL: HELLO. HELLO, EVERYONE. MY
22	NAME IS AARON NAGIEL. I'M THE PI ON THIS GRANT AND
23	THE CHIEF OF RETINA AT CHILDREN'S HOSPITAL LOS
24	ANGELES IN CALIFORNIA HERE. AND I WANT TO REALLY
25	THANK CIRM FOR THEIR ENTHUSIASTIC SUPPORT OF THIS
	11

1	WORK. AND I HOPE THAT THIS DECISION BY CIRM WILL
2	REPRESENT A HUGE MILESTONE FOR BCM PATIENTS AND
3	FAMILIES AND THE ENTIRE RETINAL DYSTROPHY COMMUNITY
4	AS WE MOVE THIS THERAPY FURTHER TOWARDS CLINICAL
5	TRIALS.
6	AS SOMEONE WHO IS DOING A LOT OF GENE
7	THERAPY WORK IN CALIFORNIA, BOTH FOR FDA APPROVED
8	(UNINTELLIGIBLE) AND OTHER TRIALS. I KNOW THAT THIS
9	WILL JUST BE ANOTHER WONDERFUL MILESTONE JUST MOVING
10	THE FIELD FORWARD AND PROUD TO HAVE A BASE IN
11	CALIFORNIA. SO THANK YOU ALL.
12	CHAIRMAN IMBASCIANI: THANK YOU. AND WE
13	HAVE ANOTHER MEMBER OF THE PUBLIC WISHING TO SPEAK.
14	MS. MANDAC: ONE MORE. PHONE NUMBER (617)
15	304-1691, IF YOU COULD PLEASE UNMUTE. SO CALLER
16	(617) 304-1691, YOU DO HAVE THE FLOOR.
17	DR. KISS: SORRY. THIS IS SZILARD KISS.
18	I'M HAVING TROUBLE UNMUTING. HOPEFULLY YOU CAN HEAR
19	ME. I'M A CO-FOUNDER OF BLUEGEN THERAPEUTICS. HAVE
20	BEEN IN THE GENE THERAPY SPACE FOR SOME TIME NOW.
21	AND I WANT TO THANK THE VOTERS OF CALIFORNIA AND THE
22	CIRM TEAM FOR SUPPORT OF THIS FIRST-IN-DISEASE
23	TREATMENT THAT CAN NOW MOVE CLOSER TO HELPING
24	PATIENTS WITH THIS DEVASTATING DISEASE. SO THANK
25	YOU SO MUCH.

12

1	CHAIRMAN IMBASCIANI: THANK YOU FOR YOUR
2	COMMENT AND YOUR WORK. ANY OTHER MEMBERS,
3	CLAUDETTE?
4	MS. MANDAC: NO. THERE ARE NO MORE HANDS
5	RAISED.
6	CHAIRMAN IMBASCIANI: OKAY. THERE'S NO
7	MEMBER OF THE PUBLIC. ANY FINAL COMMENTS FROM BOARD
8	MEMBERS? IF NOT, SCOTT, I THINK WE CAN CALL THE
9	ROLL.
10	MR. TOCHER: THE MOTION IS TO FUND
11	APPLICATION CLIN1-17090.
12	DAN BERNAL.
13	MR. BERNAL: AYE.
14	MR. TOCHER: LEONDRA CLARK-HARVEY.
15	DR. CLARK-HARVEY: AYE.
16	MR. TOCHER: ANNE-MARIE DULIEGE.
17	DR. DULIEGE: AYE.
18	MR. TOCHER: YSABEL DURON.
19	MS. DURON: YES.
20	MR. TOCHER: MARK FISCHER-COLBRIE.
21	DR. FISCHER-COLBRIE: YES.
22	MR. TOCHER: ELENA FLOWERS.
23	DR. FLOWERS: YES.
24	MR. TOCHER: DAVID HIGGINS.
25	DR. HIGGINS: YES.
	13

1	MR. TOCHER: VITO IMBASCIANI.
2	CHAIRMAN IMBASCIANI: YES.
3	MR. TOCHER: RICH LAJARA.
4	MR. LAJARA: YES.
5	MR. TOCHER: CHRISTINE MIASKOWSKI.
6	DR. MIASKOWSKI: YES.
7	MR. TOCHER: ADRIANA PADILLA.
8	DR. PADILLA: YES.
9	MR. TOCHER: JOE PANETTA.
10	MR. PANETTA: YES.
11	MR. TOCHER: MARVIN SOUTHARD.
12	DR. SOUTHARD: YES.
13	MR. TOCHER: YAEL WYTE.
14	DR. WYTE: YES.
15	MR. TOCHER: ARE THERE ANY BOARD MEMBERS
16	WHO JOINED THAT I HAVE NOT CALLED? NO. WELL, IN
17	THAT EVENT, THE MOTION CARRIES UNANIMOUSLY.
18	CHAIRMAN IMBASCIANI: WELL, THANK YOU VERY
19	MUCH. THANKS, SCOTT, FOR VOTE. THANK YOU, HAYLEY,
20	FOR THE PRESENTATION.
21	ARE THERE ANY COMMENTS FROM ANYONE ON THE
22	ARS PROCESS?
23	VICE CHAIR BONNEVILLE: VITO, I JUST
24	WANTED TO MAKE A COMMENT. THIS WAS OUR LAST CLIN
25	AWARD UNDER THE CURRENT PROGRAM ANNOUNCEMENT, AND
	14
	L

1	EVERYTHING MOVING FORWARD WILL BE UNDER OUR NEW
2	STRATEGIC ALLOCATION FRAMEWORK. AND SO I THINK
3	THAT'S A REALLY EXCITING THING TO LOOK FORWARD TO.
4	SO THANKS TO THE TEAM.
5	CHAIRMAN IMBASCIANI: GREAT. THANK YOU,
6	MARIA. WE REACHED A MILESTONE.
7	NO COMMENTS ON THE PROCESS. ANY MEMBER OF
8	THE PUBLIC WANT TO SAY ANYTHING TO THE MEMBERS OF
9	THIS COMMITTEE, WHETHER IT'S ON THE AGENDA TODAY OR
10	NOT? AND I SEE NO HANDS UP.
11	OKAY. SO I WANT TO THANK THE MEMBERS OF
12	THE BOARD FOR PARTICIPATING IN THIS MEETING TODAY.
13	AND WE'RE GOING TO ADJOURN AND CONVENE AGAIN IN
14	SEPTEMBER. THERE IS NO ARS MEETING IN JUNE. OKAY.
15	THANK YOU.
16	(THE MEETING WAS THEN ADJOURNED AT 9:17 A.M.)
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