

ICMJE DISCLOSURE FORM

Date: 10/11/2023

Your Name: Jasmina Cehajic-Kapetanovic

Manuscript Title: Gene therapy for choroideremia using an adeno-associated viral vector encoding Rab-escort protein 1: the REGENERATE open label Phase 2 clinical trial

Manuscript Number (if known): [Click or tap here to enter text.]

In the interest of transparency, we ask you to disclose all relationships/activities/interests listed below that are related to the content of your manuscript. "Related" means any relation with for-profit or not-for-profit third parties whose interests may be affected by the content of the manuscript. Disclosure represents a commitment to transparency and does not necessarily indicate a bias. If you are in doubt about whether to list a relationship/activity/interest, it is preferable that you do so.

The author's relationships/activities/interests should be defined broadly. For example, if your manuscript pertains to the epidemiology of hypertension, you should declare all relationships with manufacturers of antihypertensive medication, even if that medication is not mentioned in the manuscript.

In item #1 below, report all support for the work reported in this manuscript without time limit. For all other items, the time frame for disclosure is the past 36 months.

	Name all entities with whom you have this relationship or indicate none (add rows as needed)	Specifications/Comments (e.g., if payments were made to you or to your institution)						
Time frame: Since the initial planning of the work								
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2	Grants or contracts from any entity (if not indicated in item #1 above).	<input type="checkbox"/> None <table border="1" style="width: 100%; border-collapse: collapse;"> <tr> <td style="width: 30%;">Fight for Sight</td> <td>Studentship providing funds to support a PhD research project at my host institution (University of Oxford) developing optogenetic therapy with CRISPR-assisted activation of rhodopsin</td> </tr> <tr> <td>MRC Research Grant MR/X013189/1</td> <td>Grant providing funds to support a research project at my host institution (University of Oxford) developing a surgical procedure using a robot to optimise delivery of gene therapies to retinal ganglion cells in non-human primates (NHPs)</td> </tr> <tr> <td>MRC Clinician Scientist Fellowship MR/V029762/1</td> <td>Fellowship supporting my post and providing funds to support a research programme at my</td> </tr> </table>	Fight for Sight	Studentship providing funds to support a PhD research project at my host institution (University of Oxford) developing optogenetic therapy with CRISPR-assisted activation of rhodopsin	MRC Research Grant MR/X013189/1	Grant providing funds to support a research project at my host institution (University of Oxford) developing a surgical procedure using a robot to optimise delivery of gene therapies to retinal ganglion cells in non-human primates (NHPs)	MRC Clinician Scientist Fellowship MR/V029762/1	Fellowship supporting my post and providing funds to support a research programme at my
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			host institution (University of Oxford) developing robotic surgery for optimisation of optogenetic therapies for treatment of inherited retinal disease
3	Royalties or licenses	<input checked="" type="checkbox"/> None	
4	Consulting fees	<input checked="" type="checkbox"/> None	
5	Payment or honoraria for lectures, presentations, speakers bureaus, manuscript writing or educational events	<input checked="" type="checkbox"/> None	
6	Payment for expert testimony	<input checked="" type="checkbox"/> None	
7	Support for attending meetings and/or travel	<input checked="" type="checkbox"/> None	
8	Patents planned, issued or pending	<input checked="" type="checkbox"/> None	
9	Participation on a Data Safety	<input checked="" type="checkbox"/> None	

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	Monitoring Board or Advisory Board	<input type="text"/>	<input type="text"/>
		<input type="text"/>	<input type="text"/>
		<input type="text"/>	<input type="text"/>
10	Leadership or fiduciary role in other board, society, committee or advocacy group, paid or unpaid	<input checked="" type="checkbox"/> None	
		<input type="text"/>	<input type="text"/>
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11	Stock or stock options	<input checked="" type="checkbox"/> None	
		<input type="text"/>	<input type="text"/>
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12	Receipt of equipment, materials, drugs, medical writing, gifts or other services	<input checked="" type="checkbox"/> None	
		<input type="text"/>	<input type="text"/>
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		<input type="text"/>	<input type="text"/>
13	Other financial or non-financial interests	<input checked="" type="checkbox"/> None	
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Please place an "X" next to the following statement to indicate your agreement:

I certify that I have answered every question and have not altered the wording of any of the questions on this form.

ICMJE DISCLOSURE FORM

Date: 10/16/2023

Your Name: Marco Bellini

Manuscript Title: Gene therapy for choroideremia using an adeno-associated viral vector encoding Rab-escort protein 1: the REGENERATE open label Phase 2 clinical trial

Manuscript Number (if known): [Click or tap here to enter text.]

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11	Stock or stock options	<input checked="" type="checkbox"/> None	
12	Receipt of equipment, materials, drugs, medical writing, gifts or other services	<input checked="" type="checkbox"/> None	
13	Other financial or non-financial interests	<input checked="" type="checkbox"/> None	

Please place an "X" next to the following statement to indicate your agreement:

I certify that I have answered every question and have not altered the wording of any of the questions on this form.

ICMJE DISCLOSURE FORM

Date: 10/10/2023

Your Name: Laura Taylor

Manuscript Title: Gene therapy for choroideremia using an adeno-associated viral vector encoding Rab-escort protein 1: the REGENERATE open label Phase 2 clinical trial

Manuscript Number (if known): [Click or tap here to enter text.]

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		NIHR Oxford Biomedical Research Centre	Preparatory research career fellowship supporting my post and providing funds to support a research project at my host institution (Oxford University Hospitals NHS Foundation Trust) investigating reproducible and reliable visual function tests as clinical trial endpoints						
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		<input type="text"/>	<input type="text"/>
		<input type="text"/>	<input type="text"/>
		<input type="text"/>	<input type="text"/>

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Date: 10/10/2023

Your Name: Imran Yusuf

Manuscript Title: Gene therapy for choroideremia using an adeno-associated viral vector encoding Rab-escort protein 1: the REGENERATE open label Phase 2 clinical trial

Manuscript Number (if known): [Click or tap here to enter text.]

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9	Participation on a Data Safety Monitoring Board or Advisory Board	<input checked="" type="checkbox"/> None <table border="1" style="width: 100%; border-collapse: collapse;"> <tr><td style="width: 50%; height: 20px;"></td><td style="width: 50%;"></td></tr> <tr><td style="height: 20px;"></td><td></td></tr> <tr><td style="height: 20px;"></td><td></td></tr> </table>							
10	Leadership or fiduciary role in other board,	<input checked="" type="checkbox"/> None <table border="1" style="width: 100%; border-collapse: collapse;"> <tr><td style="width: 50%; height: 20px;"></td><td style="width: 50%;"></td></tr> </table>							

		Name all entities with whom you have this relationship or indicate none (add rows as needed)	Specifications/Comments (e.g., if payments were made to you or to your institution)
	society, committee or advocacy group, paid or unpaid		
11	Stock or stock options	<input checked="" type="checkbox"/> None	
12	Receipt of equipment, materials, drugs, medical writing, gifts or other services	<input checked="" type="checkbox"/> None	
13	Other financial or non-financial interests	<input checked="" type="checkbox"/> None	

Please place an "X" next to the following statement to indicate your agreement:

I certify that I have answered every question and have not altered the wording of any of the questions on this form.

ICMJE DISCLOSURE FORM

Date: 10/13/2023

Your Name: Taha Soomro

Manuscript Title: Gene therapy for choroideremia using an adeno-associated viral vector encoding Rab-escort protein 1: the REGENERATE open label Phase 2 clinical trial

Manuscript Number (if known): [Click or tap here to enter text.]

In the interest of transparency, we ask you to disclose all relationships/activities/interests listed below that are related to the content of your manuscript. "Related" means any relation with for-profit or not-for-profit third parties whose interests may be affected by the content of the manuscript. Disclosure represents a commitment to transparency and does not necessarily indicate a bias. If you are in doubt about whether to list a relationship/activity/interest, it is preferable that you do so.

The author's relationships/activities/interests should be defined broadly. For example, if your manuscript pertains to the epidemiology of hypertension, you should declare all relationships with manufacturers of antihypertensive medication, even if that medication is not mentioned in the manuscript.

In item #1 below, report all support for the work reported in this manuscript without time limit. For all other items, the time frame for disclosure is the past 36 months.

		Name all entities with whom you have this relationship or indicate none (add rows as needed)	Specifications/Comments (e.g., if payments were made to you or to your institution)						
Time frame: Since the initial planning of the work									
1	All support for the present manuscript (e.g., funding, provision of study materials, medical writing, article processing charges, etc.) No time limit for this item.	<input type="checkbox"/> None	<table border="1" style="width: 100%; border-collapse: collapse;"> <tr> <td style="width: 50%; padding: 5px;">NIHR Efficacy and Mechanism Evaluation (EME) Award 12/66/35</td> <td style="width: 50%; padding: 5px;">Funds supporting the REGENERATE trial were apportioned to the study sponsor (University of Oxford) and participating clinical sites (Oxford Eye Hospital; Moorfields Eye Hospital) as set out in the funding application</td> </tr> <tr> <td style="height: 20px;"></td> <td></td> </tr> <tr> <td colspan="2" style="text-align: center; font-size: small;">Click the tab key to add additional rows.</td> </tr> </table>	NIHR Efficacy and Mechanism Evaluation (EME) Award 12/66/35	Funds supporting the REGENERATE trial were apportioned to the study sponsor (University of Oxford) and participating clinical sites (Oxford Eye Hospital; Moorfields Eye Hospital) as set out in the funding application			Click the tab key to add additional rows.	
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Time frame: past 36 months									
2	Grants or contracts from any entity (if not indicated in item #1 above).	<input type="checkbox"/> None	<table border="1" style="width: 100%; border-collapse: collapse;"> <tr> <td style="width: 50%; padding: 5px;">Sir Michael Uren Fund</td> <td style="width: 50%; padding: 5px;">Funding my research fellow role under Professor Lyndon Da Cruz</td> </tr> <tr> <td style="height: 20px;"></td> <td></td> </tr> </table>	Sir Michael Uren Fund	Funding my research fellow role under Professor Lyndon Da Cruz				
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3	Royalties or licenses	<input checked="" type="checkbox"/> None	<table border="1" style="width: 100%; border-collapse: collapse;"> <tr> <td style="width: 50%; height: 20px;"></td> <td style="width: 50%;"></td> </tr> <tr> <td style="height: 20px;"></td> <td></td> </tr> </table>						

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4	Consulting fees	<input checked="" type="checkbox"/> None <table border="1" style="width: 100%; border-collapse: collapse;"> <tr><td style="width: 50%; height: 15px;"></td><td style="width: 50%; height: 15px;"></td></tr> <tr><td style="height: 15px;"></td><td style="height: 15px;"></td></tr> <tr><td style="height: 15px;"></td><td style="height: 15px;"></td></tr> </table>							
5	Payment or honoraria for lectures, presentations, speakers bureaus, manuscript writing or educational events	<input checked="" type="checkbox"/> None <table border="1" style="width: 100%; border-collapse: collapse;"> <tr><td style="width: 50%; height: 15px;"></td><td style="width: 50%; height: 15px;"></td></tr> <tr><td style="height: 15px;"></td><td style="height: 15px;"></td></tr> <tr><td style="height: 15px;"></td><td style="height: 15px;"></td></tr> </table>							
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7	Support for attending meetings and/or travel	<input checked="" type="checkbox"/> None <table border="1" style="width: 100%; border-collapse: collapse;"> <tr><td style="width: 50%; height: 15px;"></td><td style="width: 50%; height: 15px;"></td></tr> <tr><td style="height: 15px;"></td><td style="height: 15px;"></td></tr> <tr><td style="height: 15px;"></td><td style="height: 15px;"></td></tr> </table>							
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9	Participation on a Data Safety Monitoring Board or Advisory Board	<input checked="" type="checkbox"/> None <table border="1" style="width: 100%; border-collapse: collapse;"> <tr><td style="width: 50%; height: 15px;"></td><td style="width: 50%; height: 15px;"></td></tr> <tr><td style="height: 15px;"></td><td style="height: 15px;"></td></tr> <tr><td style="height: 15px;"></td><td style="height: 15px;"></td></tr> </table>							
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		Name all entities with whom you have this relationship or indicate none (add rows as needed)	Specifications/Comments (e.g., if payments were made to you or to your institution)
11	Stock or stock options	<input checked="" type="checkbox"/> None	
12	Receipt of equipment, materials, drugs, medical writing, gifts or other services	<input checked="" type="checkbox"/> None	
13	Other financial or non-financial interests	<input checked="" type="checkbox"/> None	

Please place an "X" next to the following statement to indicate your agreement:

I certify that I have answered every question and have not altered the wording of any of the questions on this form.

ICMJE DISCLOSURE FORM

Date: 10/26/2021

Your Name: Lyndon da Cruz

Manuscript Title: Gene therapy for choroideremia using an adeno-associated viral vector encoding Rab-escort protein 1: the REGENERATE open label Phase 2 clinical trial

Manuscript Number (if known): [Click or tap here to enter text.]

In the interest of transparency, we ask you to disclose all relationships/activities/interests listed below that are related to the content of your manuscript. "Related" means any relation with for-profit or not-for-profit third parties whose interests may be affected by the content of the manuscript. Disclosure represents a commitment to transparency and does not necessarily indicate a bias. If you are in doubt about whether to list a relationship/activity/interest, it is preferable that you do so.

The author's relationships/activities/interests should be defined broadly. For example, if your manuscript pertains to the epidemiology of hypertension, you should declare all relationships with manufacturers of antihypertensive medication, even if that medication is not mentioned in the manuscript.

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		Name all entities with whom you have this relationship or indicate none (add rows as needed)	Specifications/Comments (e.g., if payments were made to you or to your institution)						
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<small>Click the tab key to add additional rows.</small>									
Time frame: past 36 months									
2	Grants or contracts from any entity (if not indicated in item #1 above).	<input type="checkbox"/> None	<table border="1" style="width: 100%; border-collapse: collapse;"> <tr> <td style="width: 50%; padding: 5px;">Tenpoint Therapeutics</td> <td style="width: 50%;"></td> </tr> <tr> <td style="padding: 5px;">The Sir Michael Uren Foundation</td> <td></td> </tr> <tr> <td style="height: 20px;"></td> <td></td> </tr> </table>	Tenpoint Therapeutics		The Sir Michael Uren Foundation			
Tenpoint Therapeutics									
The Sir Michael Uren Foundation									
3	Royalties or licenses	<input checked="" type="checkbox"/> None	<table border="1" style="width: 100%; border-collapse: collapse;"> <tr> <td style="width: 50%; height: 20px;"></td> <td style="width: 50%;"></td> </tr> <tr> <td style="height: 20px;"></td> <td></td> </tr> <tr> <td style="height: 20px;"></td> <td></td> </tr> </table>						

		Name all entities with whom you have this relationship or indicate none (add rows as needed)	Specifications/Comments (e.g., if payments were made to you or to your institution)
4	Consulting fees	<input type="checkbox"/> None	
		Tenpoint Therapeutics	Payment to me
5	Payment or honoraria for lectures, presentations, speakers bureaus, manuscript writing or educational events	<input type="checkbox"/> None	
		DORC	Payment to me
6	Payment for expert testimony	<input checked="" type="checkbox"/> None	
7	Support for attending meetings and/or travel	<input checked="" type="checkbox"/> None	
8	Patents planned, issued or pending	<input checked="" type="checkbox"/> None	
9	Participation on a Data Safety Monitoring Board or Advisory Board	<input checked="" type="checkbox"/> None	
10	Leadership or fiduciary role in other board, society, committee, or advocacy group, paid or unpaid	<input checked="" type="checkbox"/> None	

		Name all entities with whom you have this relationship or indicate none (add rows as needed)	Specifications/Comments (e.g., if payments were made to you or to your institution)
11	Stock or stock options	<input type="checkbox"/> None	
		Tenpoint Therapeutics	Share of comapny
12	Receipt of equipment, materials, drugs, medical writing, gifts or other services	<input checked="" type="checkbox"/> None	
13	Other financial or non-financial interests	<input checked="" type="checkbox"/> None	

Please place an "X" next to the following statement to indicate your agreement:

I certify that I have answered every question and have not altered the wording of any of the questions on this form.

ICMJE DISCLOSURE FORM

Date: 10/12/2023

Your Name: Robert MacLaren

Manuscript Title: Gene therapy for choroideremia using an adeno-associated viral vector encoding Rab-escort protein 1: the REGENERATE open label Phase 2 clinical trial

Manuscript Number (if known): [Click or tap here to enter text.]

In the interest of transparency, we ask you to disclose all relationships/activities/interests listed below that are related to the content of your manuscript. "Related" means any relation with for-profit or not-for-profit third parties whose interests may be affected by the content of the manuscript. Disclosure represents a commitment to transparency and does not necessarily indicate a bias. If you are in doubt about whether to list a relationship/activity/interest, it is preferable that you do so.

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Time frame: past 36 months											
2	Grants or contracts from any entity (if not indicated in item #1 above).	<input type="checkbox"/> None	<table border="1" style="width: 100%; border-collapse: collapse;"> <tr> <td style="width: 50%;">Royal College of Surgeons of Edinburgh</td> <td style="width: 50%;">Grant funding a research project at my host institution (University of Oxford) to optimise delivery of a CRISPR treatment for USH2A-associated retinitis pigmentosa</td> </tr> <tr> <td>Royal College of Surgeons of Edinburgh</td> <td>Grant funding a research project at my host institution (University of Oxford) to develop a CRISPR treatment for USH2A-associated retinitis pigmentosa</td> </tr> <tr> <td>NIHR Oxford Biomedical Research Centre</td> <td>Programme funding translational research in gene and cell therapy at my host institution (University of Oxford)</td> </tr> <tr> <td>NIHR Research for Patient Benefit (RfPB) NIHR202821</td> <td>Funds supporting a cross-sectional study to assess the clinical utility of modern visual</td> </tr> </table>	Royal College of Surgeons of Edinburgh	Grant funding a research project at my host institution (University of Oxford) to optimise delivery of a CRISPR treatment for USH2A-associated retinitis pigmentosa	Royal College of Surgeons of Edinburgh	Grant funding a research project at my host institution (University of Oxford) to develop a CRISPR treatment for USH2A-associated retinitis pigmentosa	NIHR Oxford Biomedical Research Centre	Programme funding translational research in gene and cell therapy at my host institution (University of Oxford)	NIHR Research for Patient Benefit (RfPB) NIHR202821	Funds supporting a cross-sectional study to assess the clinical utility of modern visual
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			function assessments in patients with inherited retinal disease were apportioned to the study sponsor (University of Oxford) and participating clinical site (Oxford Eye Hospital) as set out in the funding application
		MRC Research Grant MR/V027557/1	Grant providing funds to support a research project at my host institution (University of Oxford) developing a novel gene therapy combining RNA interference and gene supplementation to treat autosomal dominant retinitis pigmentosa
		Retina UK and Macular Society	Studentship providing funds to support a PhD research project at my host institution (University of Oxford) developing CRISPR gene therapy for Stargardt disease
		Royal College of Surgeons of Edinburgh	Grant funding a research project at my host institution (University of Oxford) to develop CRISPR delivery strategies for the treatment of inherited retinal diseases
		University of Oxford	Grant providing funds to support a research project at my host institution (University of Oxford) developing CRISPR gene therapy for Stargardt disease
		NIHR	Senior Investigator award supporting research activities at my host institution (University of Oxford)
3	Royalties or licenses	<input type="checkbox"/> None	
		University of Oxford	Robert MacLaren is a named investigator on a patent owned by the university of Oxford
4	Consulting fees	<input checked="" type="checkbox"/> None	
5	Payment or honoraria for lectures, presentations, speakers bureaus, manuscript writing or educational events	<input checked="" type="checkbox"/> None	

		Name all entities with whom you have this relationship or indicate none (add rows as needed)	Specifications/Comments (e.g., if payments were made to you or to your institution)
6	Payment for expert testimony	<input checked="" type="checkbox"/> None	
7	Support for attending meetings and/or travel	<input checked="" type="checkbox"/> None	
8	Patents planned, issued or pending	<input type="checkbox"/> None	
		WO/2022/208084	Gene therapy using vectors that express a Cadherin-related family member 1 (CDHR1) polypeptide
		WO/2020/084319	A method for gene therapy administering a transgene for expression in the subject and a mirtron to silence expression of a target gene in the subject
		WO/2020/084318	A method for gene therapy administering a mirtron to silence expression of a target gene expressed in the retina
9	Participation on a Data Safety Monitoring Board or Advisory Board	<input checked="" type="checkbox"/> None	
10	Leadership or fiduciary role in other board, society, committee or advocacy group, paid or unpaid	<input checked="" type="checkbox"/> None	
11	Stock or stock options	<input checked="" type="checkbox"/> None	
12	Receipt of equipment, materials, drugs, medical writing, gifts or other services	<input checked="" type="checkbox"/> None	

	Name all entities with whom you have this relationship or indicate none (add rows as needed)	Specifications/Comments (e.g., if payments were made to you or to your institution)						
13 Other financial or non-financial interests	<input checked="" type="checkbox"/> None <table border="1" data-bbox="386 260 1516 361"> <tr><td> </td><td> </td></tr> <tr><td> </td><td> </td></tr> <tr><td> </td><td> </td></tr> </table>							

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