

ICMJE DISCLOSURE FORM

Date: September 4, 2024

Your Name: Rebecca Koch

Manuscript Title: Expanding therapeutic options for Pompe disease: a new small molecule inhibitor of glycogen synthase 1 (GYS1) shows preclinical promise in Pompe disease

Manuscript number (if known): ATM-24-135

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 following questions apply to the author’s relationships/activities/interests as they relate to the current manuscript only.

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 checked="" type="checkbox"/> None	
Time frame: past 36 months			
2	Grants or contracts from any entity (if not indicated in item #1 above).	<input checked="" type="checkbox"/> None	
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 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	
13	Other financial or non-financial interests	<input checked="" type="checkbox"/> None	

Please summarize the above conflict of interest in the following box:

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: September 5, 2024

Your Name: Benjamin Cocanougher

Manuscript Title: Expanding therapeutic options for Pompe disease: a new small molecule inhibitor of glycogen synthase 1 (GYS1) shows preclinical promise in Pompe disease

Manuscript number (if known): ATM-24-135

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3	Royalties or licenses	<input checked="" type="checkbox"/> None	

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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	
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13	Other financial or non-financial interests	<input checked="" type="checkbox"/> None	

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ICMJE DISCLOSURE FORM

Date: September 5, 2024

Your Name: Jeong-A Lim

Manuscript Title: Expanding therapeutic options for Pompe disease: a new small molecule inhibitor of glycogen synthase 1 (GYS1) shows preclinical promise in Pompe disease

Manuscript number (if known): ATM-24-135

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13	Other financial or non-financial interests	<input checked="" type="checkbox"/> None	

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None.

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ICMJE DISCLOSURE FORM

Date: October 1, 2024

Your Name: Nina Raben

Manuscript Title: Expanding therapeutic options for Pompe disease: a new small molecule inhibitor of glycogen synthase 1 (GYS1) shows preclinical promise in Pompe disease

Manuscript number (if known): ATM-24-135

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Time frame: past 36 months			
2	Grants or contracts from any entity (if not indicated in item #1 above).	None	
3	Royalties or licenses	None	
4	Consulting fees	None	

5	Payment or honoraria for lectures, presentations, speakers bureaus, manuscript writing or educational events	None	
6	Payment for expert testimony	None	
7	Support for attending meetings and/or travel	None	
8	Patents planned, issued or pending	None	
9	Participation on a Data Safety Monitoring Board or Advisory Board	None	
10	Leadership or fiduciary role in other board, society, committee or advocacy group, paid or unpaid	None	
11	Stock or stock options	None	
12	Receipt of equipment, materials, drugs, medical writing, gifts or other services	None	
13	Other financial or non-financial interests	M6P Therapeutics	Nina Raben is an employee of M6P Therapeutics; the company was not involved in the research described in this editorial

Please summarize the above conflict of interest in the following box:

N.R. is an employee of M6P Therapeutics; the company was not involved in the research described in this editorial.

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

 X 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: October 1, 2024

Your Name: Priya Kishnani, MD

Manuscript Title: Expanding therapeutic options for Pompe disease: a new small molecule inhibitor of glycogen synthase 1 (GYS1) shows preclinical promise in Pompe disease

Manuscript number (if known): ATM-24-135

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Time frame: past 36 months			
2	Grants or contracts from any entity (if not indicated in item #1 above).	Sanofi Genzyme	Priya S. Kishnani has received research/grant support
		Amicus Therapeutics	Priya S. Kishnani has received research/grant support
3	Royalties or licenses	<u> X </u> None	
4	Consulting fees	Sanofi Genzyme	Priya S. Kishnani has received consulting fees
		Amicus Therapeutics	Priya S. Kishnani has received consulting fees

		Asklepios Biopharmaceutical (AskBio)	Priya S. Kishnani has received consulting fees
		Maze Therapeutics	Priya S. Kishnani has received consulting fees
5	Payment or honoraria for lectures, presentations, speakers bureaus, manuscript writing or educational events	Amicus Therapeutics	Priya S. Kishnani has received honoraria
		Asklepios Biopharmaceutical (AskBio)	Priya S. Kishnani has received honoraria
		Sanofi Genzyme	Priya S. Kishnani has received honoraria
		Maze Therapeutics	Priya S. Kishnani has received honoraria
6	Payment for expert testimony	<input checked="" type="checkbox"/> None	
7	Support for attending meetings and/or travel	Sanofi Genzyme	Priya S. Kishnani has received support for travel to attend meeting/Advisory Board and Conferences
		Amicus Therapeutics	Priya S. Kishnani has received support for travel to attend meetings/Advisory Board and Conferences
8	Patents planned, issued or pending	<input checked="" type="checkbox"/> None	
9	Participation on a Data Safety Monitoring Board or Advisory Board	Sanofi Genzyme	Priya S. Kishnani is a member of the Pompe and Gaucher Disease Registry Advisory Board
		Amicus Therapeutics	Priya S. Kishnani is a member of the Pompe Disease Advisory Board
		Baebies	Priya S. Kishnani is a member of the Advisory Board
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	Asklepios Biopharmaceutical (AskBio)	Priya S. Kishnani has had equity and may receive milestone payments related to that equity in the future
		Maze Therapeutics	Priya S. Kishnani has equity
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 summarize the above conflict of interest in the following box:

PSK has received research/grant support from Sanofi Genzyme and Amicus Therapeutics. PSK has received consulting fees and honoraria from Sanofi Genzyme, Amicus Therapeutics, Maze Therapeutics, and Asklepios Biopharmaceutical, Inc. (AskBio). PSK has equity with Maze Therapeutics and has held equity in Asklepios Biopharmaceuticals and may receive milestone payments related to that equity in the future. PSK is a member of an Advisory Board for Sanofi Genzyme, Amicus Therapeutics, and Baebies. PSK has received support for travel from Sanofi Genzyme and Amicus Therapeutics.

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