

#### **Instructions**

The purpose of this form is to provide readers of your manuscript with information about your other interests that could influence how they receive and understand your work. The form is designed to be completed electronically and stored electronically. It contains programming that allows appropriate data display. Each author should submit a separate form and is responsible for the accuracy and completeness of the submitted information. The form is in six parts.

# Identifying information.

## 2. The work under consideration for publication.

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### 3. Relevant financial activities outside the submitted work.

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# 4. Intellectual Property.

This section asks about patents and copyrights, whether pending, issued, licensed and/or receiving royalties.

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**Licensed:** The patent has been licensed to an entity, whether

earning royalties or not **Royalties:** Funds are coming in to you or your institution due to your

patent



Section 1.	Identifying Inform	ation					
1. Given Name (Firs Zoheb	t Name)	2. Surnar Kazi	ne (Last Nar	me)		3. Date 28-May-2017	
4. Are you the corre	sponding author?	Yes	<b>✓</b> No	Correspond Priya S. Kis	_	or's Name	
5. Manuscript Title Sustained immun- cohort	5. Manuscript Title Sustained immune tolerance induction in CRIM-negative Pompe patients: Ten years of experience from an international						
6. Manuscript Ident	ifying Number (if you kn	ow it)					
Section 2.	The Work Under Co	onsidera	tion for P	ublication			
any aspect of the sul statistical analysis, e Are there any relev If yes, please fill ou	omitted work (including tc.)? vant conflicts of intere	but not limst?  \square\  \cdot   \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot   \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot   \cdot    \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot   \cdot   \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot   \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot   \cdot  \cdot    \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot  \cdot   \qquad \qq	nited to gran Yes pelow. If yo	nts, data monitoring	board, sti	ent, commercial, private foundation, etc.) for udy design, manuscript preparation, ty press the "ADD" button to add a row.	
Name of Institution		Grant?		Non-Financial Support?	Other?	Comments	
ienzyme Corporation	(Sanofi Aventis)	<b>✓</b>				This research was supported by a grant from the Genzyme Corpration, a Sanofi Company (Cambridge, MA), and in part by the Lysosomal Disease Network (U54NS065768) is a part of the National Institutes of Health (NIH) Rare Disease Clinical Research Network (RDCRN), supported through collaboration between the NIH Office of Rare Diseases Research (ORDR) at the National Center for Advancing Translational Science (NCATS), the National Institute of Neurological Disorders and Stroke (NINDS) and National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK). The content is solely the responsibility of the authors and does not necessarily represent the official views of the National Institute of Health. The funders had no role in study design, data collection and analysis, decision to publish, or preparation of the	



Name of Institution/Company	Grant?	Personal Fees?	Non-Financial Support?	Other?	Comments	
NIH	<b>✓</b>				This research was supported by a grant from the Genzyme Corpration, a Sanofi Company (Cambridge, MA), and in part by the Lysosomal Disease Network (U54NS065768) is a part of the National Institutes of Health (NIH) Rare Disease Clinical Research Network (RDCRN), supported through collaboration between the NIH Office of Rare Diseases Research (ORDR) at the National Center for Advancing Translational Science (NCATS), the National Institute of Neurological Disorders and Stroke (NINDS) and National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK). The content is solely the responsibility of the authors and does not necessarily represent the official views of the National Institute of Health. The funders had no role in study design, data collection and analysis, decision to publish, or preparation of the manuscript.	
Section 3. Relevant financial activities outside the submitted work.						
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Section 4. Intellectual Property Patents & Copyrights  Do you have any patents, whether planned, pending or issued, broadly relevant to the work? Yes V No						



Section 5. Relationships not severed shows
Relationships not covered above
Are there other relationships or activities that readers could perceive to have influenced, or that give the appearance of potentially influencing, what you wrote in the submitted work?
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No other relationships/conditions/circumstances that present a potential conflict of interest
At the time of manuscript acceptance, journals will ask authors to confirm and, if necessary, update their disclosure statements On occasion, journals may ask authors to disclose further information about reported relationships.
Section 6. Disclosure Statement
Disclosure Statement
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Dr. Kazi reports grants from Genzyme Corporation (Sanofi Aventis), grants from NIH, during the conduct of the study; .

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Desai 1



Section 1.	Identifying Inform	nation			
1. Given Name (Fi Ankit	rst Name)	2. Surname (Last Name) Desai	3. Date 28-May-2017		
4. Are you the corresponding author?		☐ Yes ✓ No	Corresponding Author's Name Priya S. Kishnani		
5. Manuscript Title Sustained immu cohort		in CRIM-negative Pompe	oatients: Ten years of experience from an international		
6. Manuscript Ide	ntifying Number (if you kr	now it)			
Section 2.	The Work Under C	onsideration for Public	cation		
Did you or your institution <b>at any time</b> receive payment or services from a third party (government, commercial, private foundation, etc.) for any aspect of the submitted work (including but not limited to grants, data monitoring board, study design, manuscript preparation, statistical analysis, etc.)?  Are there any relevant conflicts of interest?  Yes  No					
Section 3.	Relevant financial	activities outside the s	ubmitted work.		
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Section 4.	Intellectual Prope	rty Patents & Copyrig	yhts		
Do you have any	patents, whether plan	ned, pending or issued, br	oadly relevant to the work? ☐ Yes ✓ No		

Desai 2



Section 5. Relationships not sovered above
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Are there other relationships or activities that readers could perceive to have influenced, or that give the appearance of potentially influencing, what you wrote in the submitted work?
Yes, the following relationships/conditions/circumstances are present (explain below):
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Section 6. Disclosure Statement
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Dr. Desai has nothing to disclose.

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patent

Berrier 1



Section 1.	Identifying Inform	nation				
1. Given Name (Fi Kathryn	rst Name)	2. Surname (Last Name) Berrier	3. Date 28-May-2017			
4. Are you the corresponding author?		☐ Yes ✓ No	Corresponding Author's Name Priya S. Kishnani			
5. Manuscript Title Sustained immu cohort		in CRIM-negative Pompe	patients: Ten years of experience from an international			
6. Manuscript Ider	ntifying Number (if you kr	now it)				
			_			
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	ı					
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Are there any rel	evant conflicts of intere	est? Yes ✓ No				
	I					
Section 4.	Intellectual Proper	rty Patents & Copyrig	yhts			
Do you have any patents, whether planned, pending or issued, broadly relevant to the work? Yes Vo						

Berrier 2



Section 5.	
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	relationships or activities that readers could perceive to have influenced, or that give the appearance of encing, what you wrote in the submitted work?
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Dr. Berrier has n	othing to disclose.

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Troxler 1



Section 1.	Identifying Inform	nation						
1. Given Name (Fi Robert	rst Name)	2. Surname (Last Name) Troxler		3. Date 22-February-2017				
4. Are you the cor	responding author?	Yes 🗸 No	Corresponding A	uthor's Name				
Safe and sustain CRIM-negative in	5. Manuscript Title Safe and sustained immune tolerance induction in a large international cohort of enzyme replacement therapy treated CRIM-negative infantile Pompe disease: Long-term experience 6. Manuscript Identifying Number (if you know it)							
_		e: Long-term experience	national cohort of o	nzuma ranlacament therany treated				
Section 2.	The Work Under Co	onsideration for Pub	lication					
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Name of Entity		Grant? Personal Fees?	on-Financial Support?	Comments				
Sanofi Genzyme		✓ □		The institution received less than \$1,000 to support a Pompe data registry.				
	ı							
Section 4.	Intellectual Proper	rty Patents & Copy	rights					
Do you have any	patents, whether plan	ned, pending or issued,	broadly relevant to	the work? Yes V				

Troxler 2



Section 5. Relationships not sourced above
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Dr. Troxler reports grants totaling less than \$1,000 from Sanofi Genzyme outside the submitted work.

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Wang 1



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4. Are you the corresponding author?		Yes V No	Corresponding Author's Name Priya S. Kishnani			
5. Manuscript Title Sustained immu cohort		in CRIM-negative Pompe p	patients: Ten years of experience from an international			
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			-			
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Continue 2						
Section 3.	Relevant financial	activities outside the s	ubmitted work.			
Place a check in the appropriate boxes in the table to indicate whether you have financial relationships (regardless of amount of compensation) with entities as described in the instructions. Use one line for each entity; add as many lines as you need by clicking the "Add +" box. You should report relationships that were <b>present during the 36 months prior to publication</b> .						
Are there any rel	evant conflicts of intere	est? Yes ✓ No				
	ı					
Section 4.	Intellectual Proper	ty Patents & Copyrig	hts			
Do you have any patents, whether planned, pending or issued, broadly relevant to the work? Yes Vo						

Wang 2



Section 5.	Relationships not covered above
	relationships or activities that readers could perceive to have influenced, or that give the appearance of encing, what you wrote in the submitted work?
Yes, the follo	wing relationships/conditions/circumstances are present (explain below):
✓ No other rela	tionships/conditions/circumstances that present a potential conflict of interest
	anuscript acceptance, journals will ask authors to confirm and, if necessary, update their disclosure statements rnals may ask authors to disclose further information about reported relationships.
Section 6.	Disclosure Statement
Based on the abo	ove disclosures, this form will automatically generate a disclosure statement, which will appear in the box
Dr. Wang has no	othing to disclose.

### **Evaluation and Feedback**

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Wang 3



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**Pending:** The patent has been filed but not issued **Issued:** The patent has been issued by the agency

**Licensed:** The patent has been licensed to an entity, whether earning royalties or not

Royalties: Funds are coming in to you or your institution due to your

Abdul-Rahman 1



Section 1. Identifying Inform	nation				
1. Given Name (First Name) Omar	2. Surname (Last Name) Abdul-Rahman	3. Date 20-February-2017			
4. Are you the corresponding author?	☐ Yes 🗸 No	Corresponding Author's Name Priya S. Kishnani			
5. Manuscript Title Immune tolerance induction experienc	e in CRIM-negative Pompe	e disease patients			
6. Manuscript Identifying Number (if you ki	now it)				
		_			
Section 2. The Work Under C	onsideration for Public	cation			
Did you or your institution <b>at any time</b> receive payment or services from a third party (government, commercial, private foundation, etc.) for any aspect of the submitted work (including but not limited to grants, data monitoring board, study design, manuscript preparation, statistical analysis, etc.)?  Are there any relevant conflicts of interest? Yes Vo					
Section 3. Relevant financial	activities outside the s	submitted work.			
Place a check in the appropriate boxes in the table to indicate whether you have financial relationships (regardless of amount of compensation) with entities as described in the instructions. Use one line for each entity; add as many lines as you need by clicking the "Add +" box. You should report relationships that were <b>present during the 36 months prior to publication</b> .					
Are there any relevant conflicts of inter-	est?				
Section 4. Intellectual Prope	rty Patents & Copyric	yhts			
Do you have any patents, whether planned, pending or issued, broadly relevant to the work? Yes Vo					

Abdul-Rahman 2



Section 5.			
Section 5.	Relationships not covered above		
Are there other relationships or activities that readers could perceive to have influenced, or that give the appearance of potentially influencing, what you wrote in the submitted work?			
Yes, the following relationships/conditions/circumstances are present (explain below):			
✓ No other rela	tionships/conditions/circumstances that present a potential conflict of interest		
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Section 6.	Disclosure Statement		
Based on the abo	ove disclosures, this form will automatically generate a disclosure statement, which will appear in the box		
Dr. Abdul-Rahm	an has nothing to disclose.		

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**Royalties:** Funds are coming in to you or your institution due to your

Tanpaiboon 1



Section 1. Identifying Inform	nation			
1. Given Name (First Name) Pranoot	2. Surname (Last Name) Tanpaiboon	3. Date 18-February-2017		
4. Are you the corresponding author?	☐ Yes ✓ No	Corresponding Author's Name Priya S Kishnani		
5. Manuscript Title Safe and sustained immune tolerance induction in a large international cohort of enzyme replacement therapy treated CRIM-negative infantile Pompe disease: Long-term experience 6. Manuscript Identifying Number (if you know it)				
Section 2. The Work Under C	onsideration for Public	cation		
Did you or your institution <b>at any time</b> receive payment or services from a third party (government, commercial, private foundation, etc.) for any aspect of the submitted work (including but not limited to grants, data monitoring board, study design, manuscript preparation, statistical analysis, etc.)?  Are there any relevant conflicts of interest?  Yes  No				
Section 3. Relevant financial	activities outside the s	submitted work.		
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Section 4. Intellectual Proper	rty Patents & Copyric	ghts		
Do you have any patents, whether planned, pending or issued, broadly relevant to the work? Yes Vo				

Tanpaiboon 2



Section 5.	Relationships not covered above
	elationships or activities that readers could perceive to have influenced, or that give the appearance of ncing, what you wrote in the submitted work?
Yes, the follow	wing relationships/conditions/circumstances are present (explain below):
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Section 6.	Disclosure Statement
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pharmaceution including Edition investigator of	d honoraria and travel support from Edimer, Genzyme-Sanofi and BioMarin cals. I am a Principal investigator of several pharmaceutical-sponsored clinical trials mer, Genzyme-Sanofi, BioMarin, Ultragenyx and Shire clinical trials and a coof NIH-funded research. I have not had any financial interests, including stock and payment for consulting, with any pharmaceutical company.

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Royalties: Funds are coming in to you or your institution due to your

Mendelsohn 1



Section 1.	Identifying Inform	nation		
1. Given Name (First Name) Nancy		2. Surname (Last Name) Mendelsohn	3. Date 25-May-2017	
4. Are you the corresponding author?		☐ Yes ✓ No	Corresponding Author's Name	
cohort			e patients: Ten years of experience from an int	ternationa <b>l</b>
Section 2.	The Work Under Co	onsideration for Publ	ication	
Did you or your institution <b>at any time</b> receive payment or services from a third party (government, commercial, private foundation, etc.) for any aspect of the submitted work (including but not limited to grants, data monitoring board, study design, manuscript preparation, statistical analysis, etc.)?  Are there any relevant conflicts of interest? Yes Vo				
Section 3.	Relevant financial	activities outside the	submitted work.	
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Section 4.	Intellectual Proper	rty Patents & Copyr	ights	
Do you have any patents, whether planned, pending or issued, broadly relevant to the work? Yes Vo				

Mendelsohn 2



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Royalties: Funds are coming in to you or your institution due to your

Kronn 1



Section 1.	Identifying Inform	ation		
1. Given Name (Fi David	rst Name)	2. Surname (Last Name) Kronn	3. Date 28-May-2017	
4. Are you the corresponding author?		☐ Yes ✓ No	Corresponding Author's Name Priya S. Kishnani	
5. Manuscript Title Sustained immu cohort		in CRIM-negative Pompe	patients: Ten years of experience from an international	
6. Manuscript Ider	ntifying Number (if you kn	ow it)	_	
Section 2.	The Work Under Co	onsideration for Public	cation	
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	l			
Section 3.	Relevant financial	activities outside the s	ubmitted work.	
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Are there any relevant conflicts of interest? Yes V No				
	l			
Section 4.	Intellectual Proper	ty Patents & Copyric	hts	
Do you have any patents, whether planned, pending or issued, broadly relevant to the work? Yes Vo				

Kronn 2



Section 5. Relationships not sovered above
Relationships not covered above
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Dr. Kronn has nothing to disclose.

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**Royalties:** Funds are coming in to you or your institution due to your patent

Herskovitz 1



Section 1.	Identifying Inform	nation		
1. Given Name (Fi	rst Name)	2. Surname (Last Name) Herskovitz	3. Date 27-May-2017	
4. Are you the corresponding author?		☐ Yes ✓ No	Corresponding Author's Name Priya S. Kishnani	
5. Manuscript Title Sustained immu cohort		in CRIM-negative Pompe	patients: Ten years of experience from an international	
6. Manuscript Ider	ntifying Number (if you kr	now it)		
Section 2.	The Work Under Co	onsideration for Public	ation	
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Herskovitz 2



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This section asks about patents and copyrights, whether pending, issued, licensed and/or receiving royalties.

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**Licensed:** The patent has been licensed to an entity, whether earning royalties or not

Royalties: Funds are coming in to you or your institution due to your

Inbar-Feigenberg 1



Section 1. Identifying Inform	nation			
1. Given Name (First Name) Michal	2. Surname (Last Name) Inbar-Feigenberg	3. Date 04-March-2017		
4. Are you the corresponding author?	☐ Yes 🗸 No	Corresponding Author's Name Priya S. Kishnani		
5. Manuscript Title Safe and sustained immune tolerance induction in a large international cohort of enzyme replacement therapy treated CRIM-negative infantile Pompe disease: Long-term experience 6. Manuscript Identifying Number (if you know it)				
Section 2. The Work Under C	onsideration for Public	cation		
Did you or your institution <b>at any time</b> receive payment or services from a third party (government, commercial, private foundation, etc.) for any aspect of the submitted work (including but not limited to grants, data monitoring board, study design, manuscript preparation, statistical analysis, etc.)?  Are there any relevant conflicts of interest?  Yes  No				
Section 3. Relevant financial	activities outside the s	submitted work.		
Place a check in the appropriate boxes in the table to indicate whether you have financial relationships (regardless of amount of compensation) with entities as described in the instructions. Use one line for each entity; add as many lines as you need by clicking the "Add +" box. You should report relationships that were <b>present during the 36 months prior to publication</b> .  Are there any relevant conflicts of interest?  Yes  No  If yes, please fill out the appropriate information below.				
Name of Entity	Grant? Personal Noi	n-Financial other? Comments		
Sanofi Genzyme	✓ □	<b>✓</b>		
Section 4. Intellectual Proper	rty Patents & Copyric	ghts		
Do you have any patents, whether plan	ned, pending or issued, br	oadly relevant to the work? Yes V No		

Inbar-Feigenberg 2



Section 5. Polationships not sovered above
Relationships not covered above
Are there other relationships or activities that readers could perceive to have influenced, or that give the appearance of potentially influencing, what you wrote in the submitted work?
Yes, the following relationships/conditions/circumstances are present (explain below):
✓ No other relationships/conditions/circumstances that present a potential conflict of interest
At the time of manuscript acceptance, journals will ask authors to confirm and, if necessary, update their disclosure statements On occasion, journals may ask authors to disclose further information about reported relationships.
Section 6. Disclosure Statement
Based on the above disclosures, this form will automatically generate a disclosure statement, which will appear in the box below.
Dr. Inbar-Feigenberg reports grants and non-financial support from Sanofi Genzyme, outside the submitted work; .

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Inbar-Feigenberg 3



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**Royalties:** Funds are coming in to you or your institution due to your patent

1

Ward-Melver



Section 1. Identifying Inform	nation			
1. Given Name (First Name) Catherine	2. Surname (Last Name) Ward-Melver	3. Date 26-May-2017		
4. Are you the corresponding author?	☐ Yes 🗸 No	Corresponding Author's Name Priya S. Kishnani, MD		
5. Manuscript Title Sustained immune tolerance induction cohort	in CRIM-negative Pompe	patients: Ten years of experience from an international		
6. Manuscript Identifying Number (if you kr	now it)			
		_		
Section 2. The Work Under C	onsideration for Public	cation		
Did you or your institution <b>at any time</b> receive payment or services from a third party (government, commercial, private foundation, etc.) for any aspect of the submitted work (including but not limited to grants, data monitoring board, study design, manuscript preparation, statistical analysis, etc.)?  Are there any relevant conflicts of interest? Yes Vo				
Section 3. Relevant financial	activities outside the s	submitted work.		
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Are there any relevant conflicts of interest? Yes V No				
Section 4. Intellectual Proper	rty Patents & Copyric	ghts		
Do you have any patents, whether planned, pending or issued, broadly relevant to the work? Yes Vo				

Ward-Melver 2



Section 5. Relationships not sovered above
Relationships not covered above
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Dr. Ward-Melver has nothing to disclose.

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Royalties: Funds are coming in to you or your institution due to your

Polan 1



Section 1. Identifying Inform	nation	
1. Given Name (First Name) Michelle	2. Surname (Last Name) Polan	3. Date 28-May-2017
4. Are you the corresponding author?	☐ Yes 🗸 No	Corresponding Author's Name Priya S. Kishnani
5. Manuscript Title Sustained immune tolerance induction cohort	in CRIM-negative Pompe	patients: Ten years of experience from an international
6. Manuscript Identifying Number (if you kr	now it)	
		_
Section 2. The Work Under C	onsideration for Public	cation
	g but not limited to grants, da	a third party (government, commercial, private foundation, etc.) for ta monitoring board, study design, manuscript preparation,
Section 3. Polygant financial		
Relevant financial	activities outside the s	submitted work.
of compensation) with entities as descr	ibed in the instructions. Us	ether you have financial relationships (regardless of amount se one line for each entity; add as many lines as you need by see present during the 36 months prior to publication.
Are there any relevant conflicts of interest	· ·	- p
Section 4. Intellectual Proper	rty Patents & Copyric	yhts
Do you have any patents, whether plan	ned, pending or issued, br	oadly relevant to the work? Yes 🗸 No

Polan 2



Section 5. Relationships not sovered above
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**Royalties:** Funds are coming in to you or your institution due to your

patent

Gupta 1



Section 1.	Identifying Inform	nation	
1. Given Name (Fi Punita	rst Name)	2. Surname (Last Name) Gupta	3. Date 28-May-2017
4. Are you the cor	responding author?	☐ Yes ✓ No	Corresponding Author's Name Priya S. Kishnani
5. Manuscript Title Sustained immu cohort		in CRIM-negative Pompe	oatients: Ten years of experience from an international
6. Manuscript Ider	ntifying Number (if you kr	now it)	
Section 2.	The Work Under Co	onsideration for Public	ation
any aspect of the s statistical analysis,	ubmitted work (including	but not limited to grants, da	a third party (government, commercial, private foundation, etc.) for ta monitoring board, study design, manuscript preparation,
Section 3.	Relevant financial	activities outside the s	ubmitted work.
of compensation clicking the "Ado	) with entities as descri	ibed in the instructions. Us port relationships that wer —	ether you have financial relationships (regardless of amount se one line for each entity; add as many lines as you need by e <b>present during the 36 months prior to publication</b> .
Section 4.	Intellectual Proper	rty Patents & Copyrig	phts
Do you have any	patents, whether plan	ned, pending or issued, br	oadly relevant to the work? Yes V No

Gupta 2



Section 5.	
Section 5.	Relationships not covered above
	elationships or activities that readers could perceive to have influenced, or that give the appearance of encing, what you wrote in the submitted work?
Yes, the follo	wing relationships/conditions/circumstances are present (explain below):
✓ No other rela	tionships/conditions/circumstances that present a potential conflict of interest
	anuscript acceptance, journals will ask authors to confirm and, if necessary, update their disclosure statements rnals may ask authors to disclose further information about reported relationships.
Section 6.	Disclosure Statement
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Dr. Gupta has no	othing to disclose.

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Rosenberg 1



Section 1.	Identifying Inform	nation					
1. Given Name (Fi	rst Name)	2. Surname (Last Name) Rosenberg	3. Date 28-May-2017				
4. Are you the cor	responding author?	☐ Yes ✓ No	Corresponding Author's Name Priya S. Kishnani				
5. Manuscript Title Sustained immu cohort		in CRIM-negative Pompe	patients: Ten years of experience from an international				
6. Manuscript Ider	ntifying Number (if you kr	now it)					
			-				
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Section 3.							
Section 5.	Relevant financial	activities outside the s	ubmitted work.				
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	ı						
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Do you have any	patents, whether plan	ned, pending or issued, br	oadly relevant to the work? Yes 🗸 No				

Rosenberg 2



Section 5. Relationships not covered above
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**Entity:** government agency, foundation, commercial sponsor, academic institution, etc.

**Grant:** A grant from an entity, generally [but not always] paid to your organization

**Personal Fees:** Monies paid to you for services rendered, generally honoraria, royalties, or fees for consulting, lectures, speakers bureaus, expert testimony, employment, or other affiliations

**Non-Financial Support:** Examples include drugs/equipment supplied by the entity, travel paid by the entity, writing assistance, administrative support, etc.

Other: Anything not covered under the previous three boxes

**Pending:** The patent has been filed but not issued **Issued:** The patent has been issued by the agency

**Licensed:** The patent has been licensed to an entity, whether

earning royalties or not **Royalties:** Funds are coming in to you or your institution due to your

patent



Section 1. Identifying Info	ormation				
1. Given Name (First Name) Priya	2. Surname (Last Nam Kishnani	e)		3. Date 28-May-2017	
4. Are you the corresponding author?	✓ Yes No				
5. Manuscript Title Sustained immune tolerance induc- cohort	tion in CRIM-negative Pon	npe patients: Ten	years of ex	sperience from an international	
6. Manuscript Identifying Number (if yo	ou know it)				
Section 2. The Work Unde	er Consideration for Pu	blication			
Did you or your institution <b>at any time</b> any aspect of the submitted work (inclustatistical analysis, etc.)?					tc.) for
Are there any relevant conflicts of ir		10			
If yes, please fill out the appropriate Excess rows can be removed by pre		have more than	one entity	press the "ADD" button to add a	ı row.
Name of Institution/Company	Grant? Personal Fees?	Non-Financial Support?	Other?	Comments	
Genzyme Corporation (Sanofi Aventis)			re ho Co m Di	riya S. Kishnani has received esearch/grant support and onoraria from Genzyme orporation. Priya S. Kishnani is a nember of the Pompe and Gaucher isease Registry Advisory Board for enzyme Corporation.	



Name of Institution/Company	Grant?	Personal Fees?	Non-Financial Support?	Other?	Comments
NIH	<b>✓</b>				This research was supported by a grant from the Genzyme Corpration, a Sanofi Company (Cambridge, MA), and in part by the Lysosomal Disease Netowrk (U54NS065768) is a part of the National Institutes of Health (NIH) Rare Disease Clinical Research Network (RDCRN), supported through collaboration between the NIH Office of Rare Diseases Research (ORDR) at the National Center for Advancing Translational Science (NCATS), the National Institute of Neurological Disorders and Stroke (NINDS) and National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK). The content is solely the responsibility of the authors and does not necessarily represent the official views of the National Institute of Health. The funders had no role in study design, data collection and analysis, decision to publish, or preparation of the manuscript.

Section 3. Relevant financial	activities	s outside t	the submitted	work.		
Place a check in the appropriate boxes in the table to indicate whether you have financial relationships (regardless of amount of compensation) with entities as described in the instructions. Use one line for each entity; add as many lines as you need by clicking the "Add +" box. You should report relationships that were <b>present during the 36 months prior to publication</b> .  Are there any relevant conflicts of interest?  Yes  No  If yes, please fill out the appropriate information below.						
Name of Entity	Grant?	Personal Fees?	Non-Financial Support?	Other?	Comments	
PTC Therapeutics		<b>✓</b>				
Baebies, Inc.				<b>✓</b>	Advisory Board	
Alexion Pharmaceuticals, Inc.		$\checkmark$				
Amicus Therapeutics		$\checkmark$				
Shire Pharmaceuticals	$\checkmark$	<b>✓</b>				
Synageva		<b>√</b>			FDA Avvisory Board	



Section 4. Intelle	ctual Property	Patents & Co	opyrights			
Do you have any patents, If yes, please fill out the ap Excess rows can be remov	opropriate informat	ion below. If y	•		✓ Yes	row.
Patent?	Pending?	Issued <mark>?</mark> Licer	rsed? Royalties?	Licensee?	Comments	
20150037329 (US Patent #8,809	9,282	<b>V</b>			US Patent #8,809,282, issued August 19, 2014, "Method of Reducing Titers of Antibodies Specific for a Therapeutic Agent," Inventors: Priya S. Kishnani, Surhad C. Banugaria, Dwight D, Koeberl, Sean N. Prater.	
Section 5. Relation	onships not cove	red above				
Are there other relationsh potentially influencing, w				influenced, or th	at give the appearance of	
Yes, the following rela	tionships/conditior	ns/circumstand	ces are present (ex	plain below):		
✓ No other relationships	s/conditions/circum	stances that p	resent a potential	conflict of intere	st	
At the time of manuscript On occasion, journals may					odate their disclosure statem ships.	ents.
Section 6. Disclos	sure Statement					
		l automatically	y generate a disclo	sure statement, v	which will appear in the box	
Dr. Kishnani reports grant conduct of the study; per Pharmaceuticals, Inc., per personal fees from Synag	sonal fees from PTC rsonal fees from Am	Therapeutics icus Therapeu	, other from Baebi itics, grants and pe	es, Inc. , personal ersonal fees from	fees from Alexion	

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#8,809,282 issued.



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