STROBE Statement—checklist of items that should be included in reports of observational studies

<table>
<thead>
<tr>
<th>Item No</th>
<th>Recommendation</th>
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<tbody>
<tr>
<td><strong>Title and abstract</strong></td>
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<tr>
<td>1 (a)</td>
<td>Indicate the study’s design with a commonly used term in the title or the abstract: Yes</td>
</tr>
<tr>
<td>1 (b)</td>
<td>Provide in the abstract an informative and balanced summary of what was done and what was found: Yes</td>
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<tr>
<td><strong>Introduction</strong></td>
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<tr>
<td>2 Background/rationale</td>
<td>Explain the scientific background and rationale for the investigation being reported: Yes</td>
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<tr>
<td><strong>Objectives</strong></td>
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<tr>
<td>3</td>
<td>State specific objectives, including any prespecified hypotheses: Yes</td>
</tr>
<tr>
<td><strong>Methods</strong></td>
<td></td>
</tr>
<tr>
<td>Study design</td>
<td>4 Present key elements of study design early in the paper: Yes</td>
</tr>
<tr>
<td>Setting</td>
<td>5 Describe the setting, locations, and relevant dates, including periods of recruitment, exposure, follow-up, and data collection: Yes</td>
</tr>
</tbody>
</table>
| Participants | 6 (a) *Cohort study*—Give the eligibility criteria, and the sources and methods of selection of participants. Describe methods of follow-up: Yes  
*Case-control study*—Give the eligibility criteria, and the sources and methods of case ascertainment and control selection. Give the rationale for the choice of cases and controls: Yes  
*Cross-sectional study*—Give the eligibility criteria, and the sources and methods of selection of participants: No  
(b) *Cohort study*—For matched studies, give matching criteria and number of exposed and unexposed: No  
*Case-control study*—For matched studies, give matching criteria and the number of controls per case: NA |
| Variables | 7 Clearly define all outcomes, exposures, predictors, potential confounders, and effect modifiers. Give diagnostic criteria, if applicable: Yes |
| Data sources/measurement | 8* For each variable of interest, give sources of data and details of methods of assessment (measurement). Describe comparability of assessment methods if there is more than one group: Yes |
| Bias | 9 Describe any efforts to address potential sources of bias: Yes |
| Study size | 10 Explain how the study size was arrived at |
| Quantitative variables | 11 Explain how quantitative variables were handled in the analyses. If applicable, describe which groupings were chosen and why: Yes |
| Statistical methods | 12 (a) Describe all statistical methods, including those used to control for confounding: Yes  
(b) Describe any methods used to examine subgroups and interactions: Yes  
(c) Explain how missing data were addressed  
(d) *Cohort study*—If applicable, explain how loss to follow-up was addressed: No  
*Case-control study*—If applicable, explain how matching of cases and controls was addressed: NA  
*Cross-sectional study*—If applicable, describe analytical methods taking account of sampling strategy: NA  
(e) Describe any sensitivity analyses: NA |

Continued on next page
## Results

**Participants**  13*

(a) Report numbers of individuals at each stage of study—eg numbers potentially eligible, examined for eligibility, confirmed eligible, included in the study, completing follow-up, and analysed: Yes

(b) Give reasons for non-participation at each stage: NA

(c) Consider use of a flow diagram: NA

**Descriptive data**  14*

(a) Give characteristics of study participants (eg demographic, clinical, social) and information on exposures and potential confounders: Yes

(b) Indicate number of participants with missing data for each variable of interest: Yes

(c) Cohort study—Summarise follow-up time (eg, average and total amount): NA

**Outcome data**  15*

- *Cohort study*—Report numbers of outcome events or summary measures over time

- *Case-control study*—Report numbers in each exposure category, or summary measures of exposure: NA

- *Cross-sectional study*—Report numbers of outcome events or summary measures: NA

**Main results**  16

(a) Give unadjusted estimates and, if applicable, confounder-adjusted estimates and their precision (eg, 95% confidence interval). Make clear which confounders were adjusted for and why they were included: Yes

(b) Report category boundaries when continuous variables were categorized: Yes

(c) If relevant, consider translating estimates of relative risk into absolute risk for a meaningful time period: Yes

**Other analyses**  17

Report other analyses done—eg analyses of subgroups and interactions, and sensitivity analyses: Yes

---

## Discussion

**Key results**  18

Summarise key results with reference to study objectives: Yes

**Limitations**  19

Discuss limitations of the study, taking into account sources of potential bias or imprecision. Discuss both direction and magnitude of any potential bias: Yes

**Interpretation**  20

Give a cautious overall interpretation of results considering objectives, limitations, multiplicity of analyses, results from similar studies, and other relevant evidence: Yes

**Generalisability**  21

Discuss the generalisability (external validity) of the study results: No

---

## Other information

**Funding**  22

Give the source of funding and the role of the funders for the present study and, if applicable, for the original study on which the present article is based: Yes

*Give information separately for cases and controls in case-control studies and, if applicable, for exposed and unexposed groups in cohort and cross-sectional studies.

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**Note:** An Explanation and Elaboration article discusses each checklist item and gives methodological background and published examples of transparent reporting. The STROBE checklist is best used in conjunction with this article (freely available on the Web sites of PLoS Medicine at http://www.plosmedicine.org/, Annals of Internal Medicine at http://www.annals.org/, and Epidemiology at http://www.epidem.com/). Information on the STROBE Initiative is available at www.strobe-statement.org.
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<td>Kazi</td>
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4. Are you the corresponding author? □ Yes ✔ No

Corresponding Author’s Name

Priya S. Kishnani

5. Manuscript Title

Durable and sustained immune tolerance to ERT in Pompe disease with entrenched immune responses

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86821-INS-CMED-1

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Dr. Kazi reports grants from Genzyme, grants from NIH, during the conduct of the study.

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Prater
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<tr>
<td>Sean</td>
<td>Prater</td>
<td>20-April-2016</td>
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4. Are you the corresponding author?  
   - Yes  
   - No  
   ✔ No

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- Yes  
   ✔ Yes  
- No  
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   Joyce

2. Surname (Last Name)  
   Kobori

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Corresponding Author’s Name  
Priya S. Kishnani

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Dr. Kobori has nothing to disclose.

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ICMJE Form for Disclosure of Potential Conflicts of Interest

Section 1. Identifying Information

1. Given Name (First Name)  David H
2. Surname (Last Name)  Viskochil, MD
3. Date  26-April-2016
4. Are you the corresponding author?  [ ] Yes  [x] No
Corresponding Author’s Name  Priya S. Kishnani

5. Manuscript Title  Durable and sustained immune tolerance to ERT in Pompe disease with entrenched immune responses
6. Manuscript Identifying Number (if you know it)  86821-INS-CMED-1

Section 2. The Work Under Consideration for Publication

Did you or your institution at any time 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  [x] No

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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  [x] No
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David H. Viskochil, MD has nothing to disclose.

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ICMJE Form for Disclosure of Potential Conflicts of Interest

**Section 1. Identifying Information**

1. Given Name (First Name)  
   Carrie

2. Surname (Last Name)  
   Bailey

3. Date  
   22-April-2016

4. Are you the corresponding author?  
   ✔ No

5. Manuscript Title  
   Durable and sustained immune tolerance to ERT in Pompe disease with entrenched immune responses

6. Manuscript Identifying Number (if you know it)  
   86821-INS-CMED-1

**Section 2. The Work Under Consideration for Publication**

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ICMJE Form for Disclosure of Potential Conflicts of Interest

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Carrie Bailey has nothing to disclose.

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# ICMJE Form for Disclosure of Potential Conflicts of Interest

## Section 1. Identifying Information

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<th>3. Date</th>
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<td>Renuka</td>
<td>Gera</td>
<td>22-April-2016</td>
</tr>
</tbody>
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4. Are you the corresponding author?  
☐ Yes  ✔ No  

5. Manuscript Title  
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6. Manuscript Identifying Number (if you know it)  
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ICMJE Form for Disclosure of Potential Conflicts of Interest

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Dr. Gera has nothing to disclose.

Evaluation and Feedback

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Section 1. Identifying Information

1. Given Name (First Name)  
   David

2. Surname (Last Name)  
   Stockton

3. Date  
   19-April-2016

4. Are you the corresponding author?  
   ☑ Yes  ☐ No

   Corresponding Author’s Name  
   Priya S. Kishnani

5. Manuscript Title  
   Durable and sustained immune tolerance to ERT in Pompe disease with entrenched immune responses

6. Manuscript Identifying Number (if you know it)  
   86821-INS-CMED-1

Section 2. The Work Under Consideration for Publication

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Are there any relevant conflicts of interest?  
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<td>☑ Yes</td>
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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?  
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ICMJE Form for Disclosure of Potential Conflicts of Interest

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Section 6. Disclosure Statement

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Dr. Stockton reports grants from Genzyme, personal fees from Genzyme, outside the submitted work; .

Evaluation and Feedback

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ICMJE Form for Disclosure of Potential Conflicts of Interest

Section 1. Identifying Information

1. Given Name (First Name) Paul
2. Surname (Last Name) McIntosh
3. Date 21-April-2016

4. Are you the corresponding author? ☐ Yes ☑ No
Corresponding Author’s Name
Priya S. Kishnani

5. Manuscript Title
Durable and sustained immune tolerance to ERT in Pompe disease with entrenched immune responses

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PM receives travel assistance for speaking engagements as a member of Genzyme’s Patient Speakers Bureau.

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   - Report all sources of revenue paid (or promised to be paid) directly to you or your institution on your behalf over the 36 months prior to submission of the work. This should include all monies from sources with relevance to the submitted work, not just monies from the entity that sponsored the research. Please note that your interactions with the work’s sponsor that are outside the submitted work should also be listed here. If there is any question, it is usually better to disclose a relationship than not to do so.
   - For grants you have received for work outside the submitted work, you should disclose support ONLY from entities that could be perceived to be affected financially by the published work, such as drug companies, or foundations supported by entities that could be perceived to have a financial stake in the outcome. Public funding sources, such as government agencies, charitable foundations or academic institutions, need not be disclosed. For example, if a government agency sponsored a study in which you have been involved and drugs were provided by a pharmaceutical company, you need only list the pharmaceutical company.
4. **Intellectual Property.**
5. **Relationships not covered above.**
   - This section asks about patents and copyrights, whether pending, issued, licensed and/or receiving royalties.

**Definitions.**
- **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
ICMJE Form for Disclosure of Potential Conflicts of Interest

Section 1. Identifying Information

1. Given Name (First Name)  Amy
2. Surname (Last Name)  Rosenberg
3. Date  18-April-2016
4. Are you the corresponding author?  □ Yes  ✔ No
Corresponding Author’s Name  Priya S. Kishnani

5. Manuscript Title
Durable and sustained immune tolerance to ERT in Pompe disease with entrenched immune responses

6. Manuscript Identifying Number (if you know it)
86821-INS-CMED-1

Section 2. The Work Under Consideration for Publication

Did you or your institution at any time 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 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 present during the 36 months prior to publication.

Are there any relevant conflicts of interest?  □ Yes  ✔ No

Section 4. Intellectual Property -- Patents & Copyrights

Do you have any patents, whether planned, pending or issued, broadly relevant to the work?  □ Yes  ✔ No
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 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. Rosenberg has nothing to disclose.

Evaluation and Feedback

Please visit http://www.icmje.org/cgi-bin/feedback to provide feedback on your experience with completing this form.
ICMJE Form for Disclosure of Potential Conflicts of Interest

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.

1. **Identifying information.**

2. **The work under consideration for publication.**

   This section asks for information about the work that you have submitted for publication. The time frame for this reporting is that of the work itself, from the initial conception and planning to the present. The requested information is about resources that you received, either directly or indirectly (via your institution), to enable you to complete the work. Checking “No” means that you did the work without receiving any financial support from any third party -- that is, the work was supported by funds from the same institution that pays your salary and that institution did not receive third-party funds with which to pay you. If you or your institution received funds from a third party to support the work, such as a government granting agency, charitable foundation or commercial sponsor, check “Yes”.

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

   This section asks about your financial relationships with entities in the bio-medical arena that could be perceived to influence, or that give the appearance of potentially influencing, what you wrote in the submitted work. You should disclose interactions with ANY entity that could be considered broadly relevant to the work. For example, if your article is about testing an epidermal growth factor receptor (EGFR) antagonist in lung cancer, you should report all associations with entities pursuing diagnostic or therapeutic strategies in cancer in general, not just in the area of EGFR or lung cancer.

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ICMJE Form for Disclosure of Potential Conflicts of Interest

Section 1. Identifying Information

1. Given Name (First Name)  
   Priya

2. Surname (Last Name)  
   Kishnani

3. Date  
   22-April-2016

4. Are you the corresponding author?  
   Yes ✔ No

5. Manuscript Title  
   Durable and sustained immune tolerance to ERT in Pompe disease with entrenched immune responses

6. Manuscript Identifying Number (if you know it)  
   86821-INS-CMED-1

Section 2. The Work Under Consideration for Publication

Did you or your institution at any time 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.)?  
Yes ✔ No

Are there any relevant conflicts of interest?  
Yes ✔ No

If yes, please fill out the appropriate information below. If you have more than one entity press the "ADD" button to add a row. Excess rows can be removed by pressing the "X" button.

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<td>Genzyme Corporation (Sanofi Aventis)</td>
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<td>Priya S. Kishnani has received research/grant support and honoraria from Genzyme Corporation. Priya S. Kishnani is a member of the Pompe and Gaucher Disease Registry Advisory Board for Genzyme Corporation</td>
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This research was supported by a grant from the Genzyme Corporation, 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.

### Relevant financial activities outside the submitted work.

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Are there any relevant conflicts of interest?  ✔ Yes  ☐ No

If yes, please fill out the appropriate information below.

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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  ☐ No

If yes, please fill out the appropriate information below. If you have more than one entity press the "ADD" button to add a row. Excess rows can be removed by pressing the "X" button.

|----------------|----------|---------|-----------|------------|-----------|----------|

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Dr. Kishnani reports grants and personal fees from Genzyme Corporation (Sanofi Aventis), grants from NIH, during the conduct of the study; personal fees from PTC Therapeutics, other from Baebies, Inc., personal fees from Roche, personal fees from Alexion Pharmaceuticals, Inc/, personal fees from Amicus Therapeutics, grants and personal fees from Shire Pharmaceuticals, personal fees from Synageva, outside the submitted work; In addition, Dr. Kishnani has a patent 20150037329 (US Patent #8,809,282) issued.

Please visit http://www.icmje.org/cgi-bin/feedback to provide feedback on your experience with completing this form.