RESEARCH SUBJECT CONSENT FORM

<table>
<thead>
<tr>
<th>Title:</th>
<th>The Financial and Social Impact of Rare Diseases: Survey Instrument</th>
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<td>Protocol No.:</td>
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<td>Sponsor:</td>
<td>EveryLife Foundation for Rare Diseases</td>
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<td>Investigator:</td>
<td>The Lewin Group</td>
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<td>3160 Fairview Park Drive, #600</td>
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<td>Falls Church, VA, 22042</td>
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<td></td>
<td>USA</td>
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<tr>
<td>Study-Related Phone Number(s):</td>
<td>Phone Number 202-697-RARE</td>
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DETAILED RESEARCH CONSENT

You are being invited to take part in a research study. A person who takes part in a research study is called a research subject, or research participant.

In this consent form “you” generally refers to the research subject. If you are being asked as the legally authorized representative, parent, or guardian to permit the subject to take part in the research, “you” in the rest of this form generally means the research subject.

What should I know about this research?

- Someone will explain this research to you.
- This form sums up that explanation.
- Taking part in this research is voluntary. Whether you take part is up to you.
- You can choose not to take part. There will be no penalty or loss of benefits to which you are otherwise entitled.
- You can agree to take part and later change your mind. There will be no penalty or loss of benefits to which you are otherwise entitled.
- If you don’t understand, ask questions.
- Ask all the questions you want before you decide.

Why is this research being done?

The purpose of this research is to measure the economic impact that major rare diseases have on the U.S. economy, including non-medical costs and indirect costs. Living with a rare disease can significantly impact patients and families both financially and socially. To better understand these impacts, the EveryLife Foundation for Rare Diseases has partnered with the Lewin Group to develop a survey that looks at expenses incurred by families living with rare diseases. These costs are important considerations in healthcare decision-making that could impact patients’ access to medicines or other healthcare services. The results of this study will be used in discussions with policymakers to advocate for policies to improve the lives of people with rare diseases and their families.

The survey will be disseminated to as many as 20,000 people to take part in this research.

How long will I be in this research?

We expect that your taking part in this research will last about 45 minutes.

What happens to me if I agree to take part in this research?

This research relies on an online survey that does not collect personally identifiable information. Therefore, your participation is limited to answering the survey questions. The survey will be distributed via email through EveryLife Foundation’s partner organizations that indicated that
they would like to distribute our survey to their membership. The research team will not have access to your email address and you will not have direct contact with the research team. However, if you will have questions during the survey completion, you can reach out for clarification to the EveryLife Foundation via email which is included in the survey.

The survey consists of 39 questions and is formatted to best fit a computer screen so please complete the survey from a computer if possible. While you can leave in the middle of the survey and return at the place you left off, we highly encourage completing the survey in one sitting.

Data collected via this one-time online survey will be aggregated and analyzed by the research team from the Lewin Group at the Lewin Group’s Falls Church, VA office. They will calculate the average indirect costs for various expense categories. Therefore, no data for any respondent will be singled out and/or reported. The report and manuscript that will summarize the results of this study will report only aggregate numbers and is slated to be completed by the end of 2020. Once published, study findings will be available at EveryLifeFoundation.org.

**What are my responsibilities if I take part in this research?**

If you take part in this research, you will be responsible to: answer online survey. There will be no follow-ups.

**Could being in this research hurt me?**

Participation in an online survey is voluntary and has a minimal risk to you. It does not include physical risks, social risks, economic risks, or legal risks. However, you might experience discomfort answering questions about experiences of your family that are related to the rare disease management, which might be associated with psychological risks (for example, embarrassment).

You answers are confidential (i.e., no personally identifiable information is asked) and the datasets that will be downloaded from the online survey vendor’s website will be stored on the secure servers at the Lewin Group. The Lewin Group routinely works with confidential information like health claims data and have robust security measures in place. Therefore, privacy risks such as disclosure of private information is not possible as it is not collected; breach of the Lewin servers and access to the research dataset is also unlikely.

**Will it cost me money to take part in this research?**

Taking part in this research may lead to added costs to you, such as: costs of using internet.
Will being in this research benefit me?

There are no benefits to you from your taking part in this research. We cannot promise any benefits to others from your taking part in this research. However, possible benefits to others include learning more about economic and social impacts of rare diseases. The information you provide will be incorporated into a national study of the cost of rare disease being led by the EveryLife Foundation. Once published, study findings will be available to public at EveryLifeFoundation.org. The results of this study also will be used in discussions with policymakers to advocate for policies to improve the lives of people with rare diseases and their families.

What other choices do I have besides taking part in this research?

This research is not designed to diagnose, treat or prevent any disease. Your alternative is to not take part in the research.

What happens to the information collected for this research?

The research does not collect information on medical records or private person identifiable information (like your name, email address, or other identifiable data). Your private information (answers to the survey questions) will be shared with individuals and organizations that conduct or watch over this research, including:

- The research sponsor
- People who work with the research sponsor
- The Institutional Review Board (IRB) that reviewed this research

We may publish the results of this research. However, data will be aggregated and no individual answers will be singled out. The aggregate reporting ensures that your survey responses are confidential.

We protect your information (i.e., your responses) from disclosure to others to the extent required by law. We cannot promise complete secrecy.

De-identified data collected in this research might be used for future research without your consent.

Who can answer my questions about this research?

If you have questions, concerns, or complaints, or think this research has hurt you or made you sick, talk to the research team at the phone number listed above on the first page.
This research is being overseen by an Institutional Review Board (“IRB”). An IRB is a group of people who perform independent review of research studies. You may talk to them at (800) 232-9570, info@neirb.com if:

- You have questions, concerns, or complaints that are not being answered by the research team.
- You are not getting answers from the research team.
- You cannot reach the research team.
- You want to talk to someone else about the research.
- You have questions about your rights as a research subject.

What if I am injured because of taking part in this research?

There is no risk of injury or sickness because of being in this research.

Can I be removed from this research without my approval?

The person in charge of this research can remove your responses from this research without your approval. Possible reasons for removal include: answers that you provide are outside of reasonable range or are highly implausible. To limit such instances, the survey has safeguards against such entries by imposing logical validation rules within the online survey platform (e.g., the number of months one is employed in a year can range 0-12; answers above 12 will not be accepted). A vast majority of questions are multiple choice that minimizes risk of the removal from the study. To allow flexibility, the survey contains a limited number of open-ended questions that require text entry if appropriate.

We will tell you about any new information that may affect your health, welfare, or choice to stay in this research.

What happens if I agree to be in this research, but I change my mind later?

If you decide to leave the research early (i.e., by exiting the survey before the last question), there are no risks with this decision. No contact or notification of the research team is required.

Will I be paid for taking part in this research?

You will not be paid for taking part in this research.

Statement of Consent:

By clicking on the survey link and continuing to the online survey you provide your consent to participate in this study.