Objective/Purpose of the Project
Recent medical advancements have pointed to gene therapy as an emerging treatment intervention for individuals with hemophilia. To better understand knowledge and perception of gene therapy, patients with hemophilia and their caregivers were surveyed to:
- assess patients and caregivers’ knowledge of gene therapy;
- understand how and from whom they prefer to learn about their bleeding disorder and the potential of gene therapy; and
- identify knowledge gaps.

Methods
- An instrument was developed (IRB approval #AAAA001) using Likert-type scales, multiple choice, and open-ended questions. Prior to enrollment, construct and face validity of the instrument was obtained by piloting to community members; adaptations were made as necessary.
- Recruitment and access to the survey was done via a Web-based Community Research Portal, email list serve and social media postings with enrollment occurring between July-October, 2018. Target audience was the bleeding disorders community (BDC).
- Using Microsoft Excel 2016 and NVivo 12 descriptive statistics and qualitative data analysis was conducted (n=136).
- Participants were given the option to leave the survey at any time (attrition) as well as opt out answering particular questions throughout the survey. Various survey questions were formatted for multiple responses as well to allow for participants to select all options that were applicable.

Results

Community Awareness of Gene Therapy

<table>
<thead>
<tr>
<th>Source of Gene Therapy Awareness (N=130)</th>
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</thead>
<tbody>
<tr>
<td>US government agency</td>
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<tr>
<td>Public radio or television station</td>
</tr>
<tr>
<td>Local or national news station</td>
</tr>
<tr>
<td>In an educational course or class</td>
</tr>
<tr>
<td>Healthcare provider</td>
</tr>
<tr>
<td>Biotechnology or pharmaceutical company</td>
</tr>
<tr>
<td>Local patient advocacy organization</td>
</tr>
<tr>
<td>Practical or scientific publications or websites</td>
</tr>
<tr>
<td>National patient advocacy organization</td>
</tr>
</tbody>
</table>

Gene Therapy Awareness (N=135)

- Never Heard About It: 44% (n=59)
- Could Explain It: 15% (n=20)
- Unsure: 0% (n=0)
- Unsure: 35% (n=47)
- Only Know a Little: 46% (n=62)

Most participants have heard of ‘gene therapy’ but the extent of knowledge varied. When stratifying by patient vs. caregivers, the patients self-reported a higher level of knowledge (49%) while 33% of caregivers reported having at least enough knowledge to explain gene therapy.

Awareness of gene therapy vs. gene editing (N=123)

Using multiple choice, participants were asked to select the extent of knowledge they have regarding gene editing. The majority of participants (52%, n=64) stated that they had heard of gene editing, with a further 35% (n=41) stating that they had heard of ‘gene therapy’ but were unsure of what it was. Only 13% (n=16) of participants had heard of gene therapy and were able to explain it.

Concerns for a Potential Gene Therapy Treatment (N=43)

- Price of the therapy: 19% (n=8), 67% (n=29), 14% (n=6)
- Insurance coverage: 86% (n=37), 14% (n=6)
- Future therapy or long-term outcomes: 6% (n=2), 94% (n=41)

Top 5 Concerns
- What might be the side effects of a potential gene therapy? (n=102)
- Is gene therapy safe? (n=96)
- Will insurance pay for it? (n=90)
- How long could a potential gene therapy last? (n=89)
- How much could a potential gene therapy cost? (n=86)

How do you want to know about a potential gene therapy for hemophilia? (N=122)

- I don’t want to know anything: 7% (n=8)
- I want to know about the science of gene therapy research: 82% (n=101)
- I want to know what is going on at the moment: 8% (n=9)
- I want to be a part of the trial: 3% (n=4)

Expectations for a Potential Gene Therapy (N=57)

- Expected to cure now: 7% (n=4)
- Expected to cure in the future: 80% (n=46)
- Expected to temporary changes: 5% (n=3)
- Expected to improve future therapy or long-term outcomes: 7% (n=4)

Conclusion
While expectation and excitement around the potential of gene therapy is high, the study demonstrates participants’ perceptions of access to limited information around the topic as a whole, which may lead to an increase in the increase in the frequency of the choice “I don’t know enough” as a concern for a potential gene therapy. Participants express a desire to learn more about gene therapy research, specifically for some of our participants, these two technologies are indistinct. The implications of and differences between these two technologies are important for the community to understand. These results suggest clarification between these terms is warranted.

Acknowledgments
This work was supported by a grant from Spark Therapeutics. The authors would like to acknowledge the representatives of bleeding disorders organizations and patients with hemophilia A who participated in this study. This work would not have been possible without the support and dedication of all community members who took the time to complete the survey.

References

Limitations
Our survey title, “Gene Therapy and Me” may have been imprecise. The BDC may have attracted individuals with more optimistic views of or interest in, gene therapy, or perhaps implied a need to have expectations with gene therapy. Other scenarios warranting further research that participants were not so representative of individuals who are wiser of and in favor of gene therapy. Based on the research team’s interactions with the bleeding disorders community, this may have led to an under-representation of individuals with less optimistic views or interest in gene therapy.

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