Enhancing Awareness and Understanding of Gene Therapy among Rare Disease Communities: A Research-Driven Roadmap

Christopher Smith[1], Katie Burns, MBA[1], Patti Engel, RN, BSN[2], and Peter Steinberg, MBA[1]
[1] SmithSolve LLC, Morristown, NJ, USA; [2] Engage Health, Eagan, MN, USA

Background

Gene therapy is one of the most promising investigational treatment modalities for rare diseases. However, its complexity poses significant communication and educational challenges. Among the lay public, there is a general awareness and acceptance of gene therapy for rare and serious disease, but not an accurate understanding. Public and physician understanding of gene therapy is hampered by confusion over what gene therapy is, a problem exacerbated by the lack of educational resources and a universally accepted definition and nomenclature.

The gene therapy revolution is progressing rapidly, as evidenced by the recent approval of the second gene therapy treatment in Europe and multiple late-stage clinical trials around the world. The Alliance for Regenerative Medicine recently reported that 802 clinical trials for gene and cell therapies were underway in 2016, including 66 Phase 3 trials (Figure 1).[1] Such developments make this a critical time for stakeholders to unite in a collaborative effort to enhance education about gene therapy.

Figure 1: Number of cell and gene therapy clinical trials worldwide.

802 Total Clincal Trials: End of 2016
21% growth over 2015

271 Phase I	465 Phase II	66 Phase III
192 in 2015	376 in 2015	63 in 2015

Adapted from Alliance for Regenerative Medicine[1]

Objectives and Methods

This multifaceted research project assessed awareness, perceptions, and understanding of gene therapy as a potential treatment for rare diseases across various audiences.

Peer Reviewed Literature Audit
- A review and analysis of articles published in English and gathered from PubMed and other library databases by entering key search terms such as "gene therapy misconceptions" and "gene therapy education."
- The articles included in the analysis addressed the history of gene therapy and assessed public and healthcare provider knowledge and perceptions of gene therapy.

Industry Expert Interviews
- A series of 30-minute phone interviews with 12 representatives of the pharma/biotech industry and academia.
- The 10 industry representatives included employees of private and publicly traded biopharmaceutical companies with investigational gene therapies in development for the treatment of rare disorders.
- The 2 academic representatives were from research departments at major U.S. universities actively engaged in gene therapy research.

Social and Traditional Media Audit
- An audit and analysis of social and traditional media coverage of gene therapy.
- The social media audit covered 82,519 U.S.-based, English-language posts about gene therapy from August 2015 through August 2016, as captured through multiple aggregators.
- The traditional media audit covered 4,931 stories about gene therapy for rare diseases over the same time period.

Patient and Caregiver Survey
- A Web-based survey of patients and caregivers from various rare disease communities.
- The survey sample consisted of patients or parents of patients who are diagnosed with a rare disease for which gene therapy is either available or in development.

The authors initiated this research to address the needs of rare disease patient communities and to fulfill Patient Advocacy Certificate Training (PACT) requirements of the Professional Patient Advocates in Life Sciences (PPALS).

Results

Peer-Reviewed Literature Audit

An audit of selected articles from peer-reviewed literature yielded several findings including:

- A 2014 survey of 467 residents of Canada and the U.S. found widespread agreement (75%) that gene therapy "will have a positive impact on society," along with concerns about "not receiving all the appropriate information regarding the treatment."[2]
- Earlier opinion polls revealed public concerns about cloning[3], genetics researchers' potential conflicts of interest[4,5], lack of research participant compensation for investigators' discoveries[6], erosion of medical privacy[3,7,8], genetics-based discrimination[3,9], corporate exploitation of genetic technology[6], and creation of "a permanent genetic underclass."[6]
- Positive media coverage of genetic research often lacks critical commentary, disconfirming evidence, or contextual information that would allow readers to assess the validity or credibility of the research, potentially engendering unrealistic expectations about the promise of gene therapy.[10,11]

Industry/Academia Interviews

In general, the expert interviewees agreed that the typical physician does not fully understand gene therapy technology or treatment, or may not prioritize such understanding.

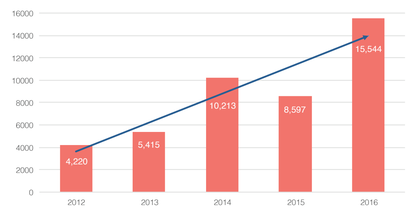
- The most frequently cited physician misconceptions about gene therapy were that it "cures the patient forever," and that lingering safety concerns or "old problems" still exist.
- While the lack of an approved gene therapy in the U.S. may be fueling such misconceptions, other potential contributing factors include the newness of genetic testing technologies, as well as the lack of educational resources, particularly for older doctors who were trained in a time when gene therapy was not discussed.

"Physicians don't yet have a good understanding of what gene therapy really is. There are a lot of methods and people are afraid that it changes the genome, when it's just delivering what's missing. The technology has also come a long way, but they don't understand how the safety has improved."

Social Media Audit

- Of 82,519 total gene therapy-related posts from Aug. 2015 to Aug. 2016, 12,868 (16%) specifically focused on "gene therapy for rare diseases."
- An analysis of posts focusing on "gene therapy for rare diseases" from 2012 to 2016 suggests an upward trajectory of conversation on this topic in parallel with increases in industry R&D efforts and clinical trial activity (Figure 2).

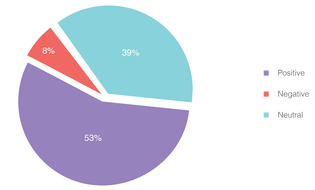
Figure 2: Volume of social media posts focusing on gene therapy for rare diseases 2012-2016.



Social Media Audit, Cont.

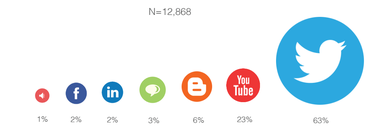
- Clinical trials, product innovation and regulatory actions were main conversation drivers, suggesting an industry-led conversation.
- Sentiment was largely positive or neutral (Figure 3).

Figure 3: Sentiment of social media conversations.



- Twitter emerged as the dominant social media channel, accounting for 63% of the volume, which generally consisted of retweets and shared links focusing on gene therapy clinical trial results and techniques. YouTube, at 23% of the volume, also drove considerable coverage, largely due to user engagement with videos offering basic information on new genetic technologies (e.g., CRISPR) and news of biotech executives (e.g., Liz Parrish of BioViva) volunteering for gene therapy trials (Figure 4).
- These findings suggest that YouTube will continue to present opportunities for deeper engagement and may emerge as a main educational "hub" for gene therapy, while Twitter will remain a powerful sharing tool.

Figure 4: Distribution of conversation among social media channels.

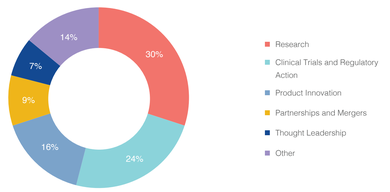


Traditional Media Audit

Of the 4,931 stories about gene therapy for rare diseases published between August 2015 and August 2016, we analyzed a sample of 466, 42% of which were positive, 4% negative, and 54% neutral.

- The audit revealed a steady increase in mainstream media coverage of gene therapy, from 237 stories in August 2015 to 703 in August 2016.
- There was a spike in coverage in the summer (June through August) of 2016, with many articles focusing on clinical trial developments (e.g., enrollment milestones), innovation (e.g., hints of a "cure for a rare and deadly disease"), and regulatory milestones (e.g., FDA or EMA approval, Breakthrough Therapy Designation).
- Media conversation drivers over the course of the year suggest an industry-led conversation, with the majority of coverage focused on topics such as new developments in laboratory research, clinical trials, regulatory actions, and product innovation (Figure 5).

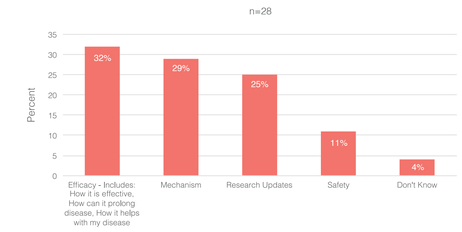
Figure 5: Traditional media conversation drivers.



Patient/Caregiver Survey

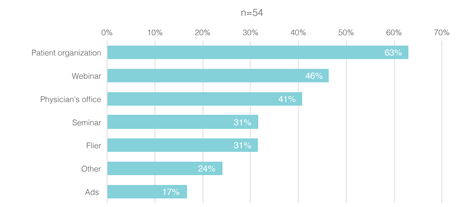
- Of the 54 survey respondents:
  - 32 (59%) were parents of a child with a rare disease
  - 16 (30%) were individuals aged 18 years or older who had been diagnosed with a rare disease
  - 5 (9%) were parents or legal guardians of persons aged 18 or older who were unable to answer for themselves
  - 1 (2%) did not indicate whether he/she was a patient or a caregiver
- When asked to rate their knowledge of gene therapy on a scale of 1 to 10 (with 1 being extremely low and 10 being extremely high), 35% of respondents gave themselves a score of 6 or higher.
- One-third (32%) of the responders said they wanted to know more about the efficacy of specific gene therapies, and nearly as many (29%) expressed interest in learning more about gene therapy mechanisms (Figure 6).

Figure 6: Types of information patients and caregivers wanted to know more about.



- Patient advocacy organizations (63%) were the most preferred source of gene therapy information (Figure 7).

Figure 7: Patients' and caregivers' most preferred sources of gene therapy information.



Conclusions

Based on our research, we submit the following recommendations for improving communication and enhancing awareness of gene therapy:

- Establish a **common nomenclature** that provides relevant, consistent, and understandable information about gene therapy.
- Foster **realistic expectations** about gene therapy by interacting with the media (and other external audiences) in a way that conveys the promise of gene therapy realistically and responsibly.
- Secure **alignment** among industry, academic centers, trade groups, government agencies, and patient advocacy groups to facilitate **consistency in educational efforts**.
- **Encourage a dialogue** among and between audiences, as opposed to simple, "top-down" dissemination of information through conventional communication channels.
- **Engage** audiences through **multiple media** (i.e., supplement printed materials with video, infographics, other interactive media).
- Provide **basic education about genetics**, based on a reasoned and critical **assessment of the health literacy needs** of the population.

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