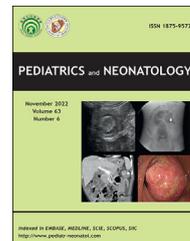


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Perspectives

Crowdfunding approach for gene therapy: Experience from the UAE

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Received Apr 7, 2022; received in revised form Jun 25, 2022; accepted Jul 7, 2022

Available online 9 September 2022

Spinal muscular atrophy (SMA) is a progressive neurological disease characterized by severe muscle weakness caused by losing spinal motor neurons.¹ It is a rare genetic disease with around 1 in 10,000 incidence rate and is caused by a deletion of the *SMN1* gene.²

Three medications have been approved for the treatment of SMA in the past 5 years, including gene therapy (onasemnogene abeparvovec), also known as Zolgensma, which changed the landscape of the disease as it targets the root cause of the illness by transferring the missing gene into human cells.³ Since its approval in 2019, only over a thousand patients worldwide have received Zolgensma despite its proven efficacy and safety and convenience as it is given only once in life. Its high price, \$2.125 million, puts it out of reach for the majority of patients, insurance companies, and countries, is a significant limiting factor for its use clinically.³

Some of our patients used an exciting approach called crowdfunding to pay for the expensive treatment cost and get the therapy. Crowdfunding is not a novel idea; it has been employed for years to raise money from the general public to fund projects.⁴ There have been early reports that Joseph Pulitzer crowdfunded a pedestal for the Statue of Liberty via the Pulitzer newspaper. In the digital era, crowdfunding has moved to online platforms, and its effect has been augmented by the influence social media has on our daily lives. Different types of crowdfunding exist; in

donation crowdfunding, funders usually support a charity project without the expectation of a monetary compensation, rather recognition in other forms is expected. A good example of this is JustGiving website. While medical crowdfunding, in particular, has been used in public health campaigns like vaccine developments, another growing area of medical crowdfunding is for treatment, either for expensive medical bills or sponsor medical treatment; these include GoFundMe, YouCaring, and many other US-based companies,⁴ with reports of an increasing number of cases registered annually in the US.⁴

Families of patients in our center afforded this expensive treatment via online crowdfunding during the hajj pilgrimage and the holy month of Ramadan. During the holy times, families managed to gather an average of 80%–90% of the total required for the therapy. Families used social media and “hashtag campaigns” to promote their case, with some families using online streaming and online live chat to highlight their child’s challenges. Only 2 of the 30 patients in our cohort had insurance coverage for their treatment, while four patients used social media to build support for their cause and end up with the treatment being covered by a celebrity or a member of the royal family. The rest of our cohort had the treatment covered via crowdfunding.

According to Bassani et al., there are 76 online medical crowdfunding platforms active worldwide, with an estimated gross of \$132 million annually.⁵ Most of the reports come from the US, mostly due to individual financial difficulties brought on by steadily increasing medical bills. Interestingly, there has been an increasing report of

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crowdfunding cases from countries with universal healthcare system. For instance, Lublóy et al., cited the lengthy legislative process to emerging therapeutics and the increasing demand for high-quality medical care as a reason for crowdfunding in Europe, which, broadly speaking, is a universal healthcare system with variability among its member countries.⁶

Crowdfunding is a viable alternative to conventional borrowing and, in theory, accessible to everyone, private individuals as well as economic entities.⁷ Due to the decentralized structure of the crowdfunding industry, determining its size is difficult. It is estimated that health care initiatives had earned more than \$132 million by October 2017. By comparison, medical-crowdfunding campaigns on GoFundMe reach a significantly wider audience. By 2018, GoFundMe has collected over \$650 million globally for medical causes by 250,000 campaigns.

Crowdfunded expensive gene therapy meets a negligible proportion of medical and health-related requirements that remain unmet. By and large, crowdfunding campaigns are initiated by younger individuals with more advanced computer knowledge. Berliner and Kenworthy claim that crowdfunding campaigns are often started by individuals with exceptional reading and writing skills. Health, social media, and technological literacy are all necessary. Furthermore, Snyder et al., claim that crowdfunding is popular among relatively affluent people of society, such as those who are technologically savvy and have extensive social networks.⁸ Indeed, big social networks contribute significantly to fundraising success; promoting initiatives through social media platforms enhances the likelihood of success.

Crowdfunding for expensive genetic treatment is a relatively new but quickly increasing phenomenon in which individuals (co-)finance their health care expenses via numerous individuals making (very modest) contributions.⁹ The popular crowdsourcing site, GoFundMe, has projects for various medical (and social) needs for patients. Medical crowdfunding's individual and systemic effects are slowly appearing, primarily in the US and Canada.

According to Saleh et al., crowdfunding for expensive genetic therapy was initially hailed as a form of digital insurance.¹⁰ Alternatively, democratizing philanthropy can be accomplished by making it accessible to everyone. Factors influencing crowdfunding success, include recipient demographic, treatment kind, campaigning nation, the campaign's fundraising target, the number of contributors, and the duration of the campaign narrative. Setting a more significant objective for expensive genetic therapy campaigns, according to Snyder et al., may help contributors understand their needs and promote the idea of deservingness. The length of the campaign story underscores the critical nature of the disease narrative to fundraising success.

The market for crowdfunded expensive gene therapy is substantial and rapidly expanding with new genetic therapies on the horizon, for example, for Duchane muscular

dystrophy, crowdfunding will become more streamlined, especially if insurer policies that do not cover genetic conditions continue. The legislative issue with crowdfunding is challenging, with no right or wrong answer, and no answer that fits all, especially that the funding is often from international sources as well as national sources. Thus, the process of setting in laws that govern this transaction should be implemented by all stakeholders, including the media that the process occurs through, not only the governmental institutions. As the market expands, proper legislation should be enacted to support crowdfunding in medical diseases. Currently, the process is decentralized and is dependent on collaborative effort.

Declaration of competing interest

The authors have no conflict of interest.

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Appendix A. Supplementary data

Supplementary data to this article can be found online at <https://doi.org/10.1016/j.pedneo.2022.07.006>.