EDITORIAL

The Role of Patient Advocacy Organizations in Advancing Human Gene Therapy

Terence R. Flotte

Editor in Chief

> *Never doubt that a small group of thoughtful, committed citizens can change the world; indeed, it's the only thing that ever has.*
>
> *—Margaret Mead*

THE RECENT SUCCESSES of gene therapy experiments in early phase clinical trials have, ironically, occurred at a time when federal funding for biomedical research has become markedly constrained, resulting in a dramatic drop in the success rates of grant applications to the National Institutes of Health. An increase in private-sector investment in gene therapy has partially filled that gap. However, the availability of such funding for any individual project or disease condition is highly variable. The particular suitability of gene therapy for monogenic disorders also means that gene therapy is very often targeted to rare diseases, which can affect the market considerations for potential investors.

In response to these trends, patient advocacy organizations and disease-based foundations have expanded their efforts to promote the development of gene therapy vectors and other molecularly targeted therapies. Pioneering organizations, such as the Cystic Fibrosis Foundation and the Muscular Dystrophy Association in the United States and the AFM Telethon in France, began such efforts in the late 1980s and early 1990s promoting basic, clinical, and translational research, as well as direct advocacy with federal funding and regulatory agencies. These missions were added to traditional roles of providing educational resources and many forms of medical and social assistance to patients and families. The role of these and other organizations has expanded in recent years including the new realms of providing pharmacy and home infusion services, and even direct investment in promising new therapies, the so-called "venture philanthropy." The role of such investments generally is to leverage private-sector investment, thereby de-risking such investments and making them more desirable for companies with a platform technology to choose "their disease."

Beginning in this December 2015 issue, *Human Gene Therapy* will periodically highlight successes and challenges faced by such organizations in a series of special commentaries. We will begin this month with a contribution from Jean-Marc Quach, Robert Campbell, and John Walsh, from the Alpha One Foundation, a nonprofit organization founded 20 years ago to promote cutting-edge research focused on finding a cure for alpha-1 antitrypsin (AAT) deficiency, a common single-gene disorder. Through a combination of academic research funding and commercial investments through its venture philanthropy arm (The Alpha One Project), the Alpha One Foundation has promoted the development of gene- and siRNA-based therapy for AAT deficiency and de-risked investments by certain specific companies who might have chosen to pursue other diseases to test their various platform technologies.

Over the coming months, we will present a number of such commentaries. It is our hope that each of these unique stories will assist with the spreading of best practices among the patient advocacy community and assist each individual contributor in raising the awareness and interest among investigators and investors alike.

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