# The Value of Patient Advocacy Groups in Rare Disease Development: *A Path to Strong Patient Engagement*

Clinical trials in therapeutic areas targeting rare diseases are riddled with operational difficulties, with patient recruitment being arguably the most challenging aspect. By definition, a rare disease occurs in fewer than 200,000 people in the US (and less than 250,000 in the EU). Patient advocacy groups (PAGs) are, therefore, vital partners for sponsors in engaging with hard-to-find patients and their caregivers.

**Why is Recruitment So Difficult?**

The most obvious reason is the simple fact that patients with rare diseases are few and far between. As a result, it is not unusual to conduct a global search just to find a small number of participants, and then it is challenging to find experienced sites that can properly manage them. But, there are other factors that hinder recruitment:

- Patients often go undiagnosed or misdiagnosed for years, with the average time to a correct diagnosis being five to seven years.
- Patients often suffer from physical and/or mental impairment, making the logistics of participation burdensome for patients and their caregivers.
- Patients and caregivers often are unaware of trials for which they may qualify.
- Protocols in these disease areas tend to be complex, increasing the burden on patients/caregivers, which, in turn, tends to suppress participation.
- Often the lack of standard care, whereby rare diseases are managed differently from site to site, opening up challenges in meeting inclusion/exclusion criteria and designing protocols.

**What do Patient Advocacy Groups Offer?**

PAGs serve as a trusted voice in a rare disease community and have a solid understanding of what is needed in the disease area across the health system. They are well placed in the disease ecosystem and maintain relationships with key opinion leaders (KOLs), principal investigators, and patients and their families. They are, in fact, often the first place that patients turn when seeking information and resources.

PAGs' role to promote collaboration between different stakeholders is fundamental to accelerate treatment access for rare disease patients. As an example, in December 2017, the US Food & Drug Administration (FDA) released draft guidance on Pediatric Rare Diseases — A Collaborative Approach for Drug Development Using Gaucher Disease as a Model – which is promoting the development of umbrella protocols involving multiple products/sponsors to minimise the number of patients exposed to a placebo. To achieve this collaboration, the role of PAGs will be critical to putting the patient at the centre of clinical development strategy.

In collaborating with PAGs and tapping their knowledge, network, and resources, sponsors can gain:

- Valuable input into study design and logistics in order to optimise the protocol from the patient's perspective and minimise its complexity
- Access to information, data, and resources
- Support in seeking research grants
- Access to KOLs
- Access to patients for focus groups
- The ability to engage with patients via the PAG's established channels which may include social media, newsletters, and support groups

Insights gleaned from the PAG and its connections can be invaluable in shaping a patient engagement strategy for research that ultimately delivers meaningful results for patients. For example, by working closely with physicians and the PAG in spinal muscular atrophy (SMA), Syneos Health was able to develop a clinical protocol that aligned patient management through different countries. The PAG then helped in raising awareness of the study among patients and families.

Sponsors can also use the insights they gain to develop patient support strategies that are effective in reducing the patient/caregiver burden.

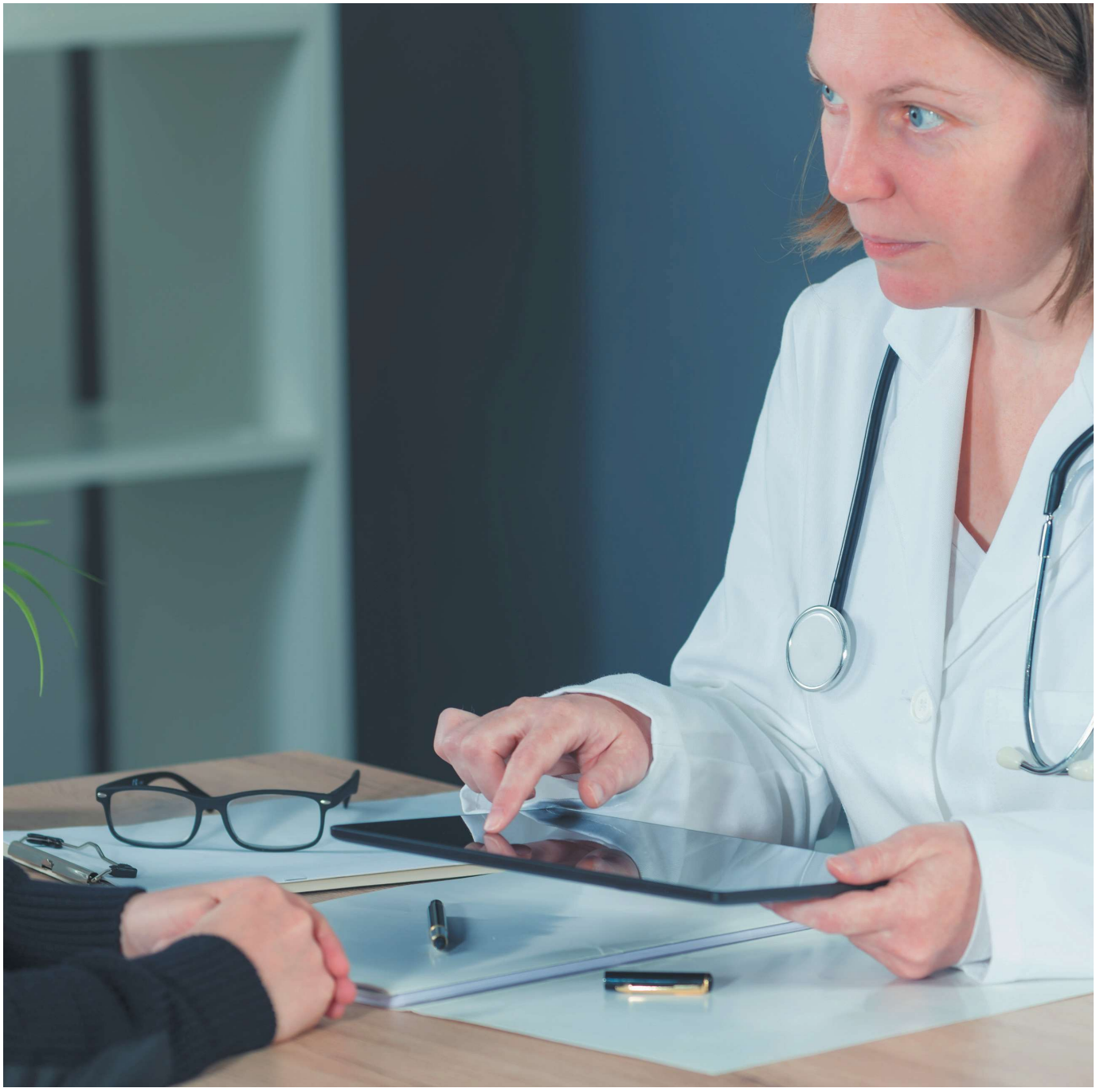
**How are Partnerships Structured?**

Sponsors and PAGs share the goal of helping patients with rare diseases access treatment; however, they could follow different paths to realising that goal. It will be important to clearly define areas of collaboration, and there are examples such as the Engagement Plan from the Patient-Centered Outcomes Research Project run by NORD that can be a useful supporting tool. In some partnerships, PAGs help sponsors gather advice from KOLs, gain support for recruitment initiatives, and review manuscripts or grants, although they remain independent. Other, more formal partnerships are designed so that risk and responsibility for the outcome of the initiative is shared. In this contractual arrangement, each party has a vested interest in the outcome, and accountability for its success is balanced. In October 2017, "Guidelines for Interactions Between Patient Advocacy Organizations and Biopharmaceutical Companies" were finalised. The piece translates the experience of one advocacy group into a best practice and defines the key principles of collaboration, as agreed upon by representatives from the rare disease industry.

**How to Approach a PAG**

The primary key to success in working with a PAG to develop a patient-centred research plan is to engage the group early so that the advice and insights the relationship yields can be applied to the protocol, message development, and recruitment plan. Companies should approach the collaboration as any other partnership by:

- **Establishing common goals so that the relationship is a win-win.** These organisations are often poorly funded and will receive value from support with research materials, educational materials, and other tools to advance their mission.
- **Defining clear responsibilities and boundaries.**
- **Promoting bi-directional learning.** Researchers and advocacy groups need to understand one another's languages, challenges, and needs.



- **Cultivating a long-term relationship.** The relationship must be part of a long-term strategy. It is not a tactical line item that can be switched on or off as a study progresses. PAGs respond better to sustained, rather than passing, interest.

By cultivating mutually beneficial relationships with the relevant PAGs, sponsors developing treatments for rare diseases can, ideally, overcome recruitment challenges by ensuring that their protocol is patient-centred and by using the connection to raise awareness of the study itself.

**What can you do if no advocacy group exists?**

Often, with very rare diseases, no patient advocacy group has yet been formed. When that's the case, sponsors can help patients and caregivers connect with one another – a step that may eventually lead to the creation of a PAG. This can be done by using social media to drive interested parties to a website where they can opt in to a community or by turning to a specialised vendor who can run a social media campaign and establish the online presence. Once the online community is established, it can serve as a key avenue of patient engagement for the sponsor.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