The Importance of Patient Advocacy

***EBR* sits down with Jennifer McGrain, Coordinator of Patient Advocacy at Zambon, to discuss the critical role of the patient advocate in understanding and addressing rare diseases**

***EBR:* What is the patient advocacy function within a pharmaceutical company and why is it important?**

Jennifer McGrain: The role of a patient advocate is to serve as a liaison between the patient community and a pharmaceutical company – it is an essential part of incorporating the patient voice into company programmes as both parties look to secure optimal outcomes for the end-users. The role addresses three key goals:

- Build relationships with the patient community to uncover their needs and concerns, better understand the unmet needs for their condition, and determine how we can help improve their experience
- Raise awareness of the disease and treatments in development with policymakers in an effort to improve patient access to therapies upon approval
- Educate the patient community about new research opportunities for their disease to ensure that they are informed

Why is patient advocacy of particularly high importance in rare diseases compared to more common diseases?

An estimated 300 million people worldwide have one or more of the 6,000 identified rare diseases (1). The unmet need in this patient population is high and extremely urgent as most rare diseases have no approved treatments. As rare diseases often affect only a handful of people located in a multitude of locations worldwide, many patients have no organised group they can rely upon for resources, emotional support, and advice. While social media has, to some extent, been helpful in connecting patients and their caregivers to others with the same rare conditions, a patient advocate can help unite these populations, giving them both a voice and direction.

Is it common for biotechnology and pharma companies to have dedicated patient advocacy personnel?

Among participants surveyed by *BioNJ*, 76% of biopharmaceutical companies had patient advocacy programmes, or planned to implement them, yet only 19% had personnel dedicated to patient advocacy. These numbers suggest that although companies place a value on patient advocacy, the resources to staff this function may be limited (2).

What situations might prompt a company in the rare disease space to incorporate the patient advocacy area?

As previously discussed, patient advocacy in rare disease communities is crucial. Working with patients and patient organisations helps the company improve disease understanding, proactively answer relevant clinical questions, and furthers an understanding of important patient-centric outcomes for a rare disease population (3). A key situation that may encourage the enhancement of patient advocacy is in clinical trials. With rare disease patients being a group limited in size, geographically disparate and heterogeneous amongst themselves, identifying patients for participation in clinical trials can be challenging (3). The support of patient advocacy to facilitate engagement can be essential in raising awareness of trials within the patient population, which may lead to faster enrolment.

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What would a strong collaboration process with rare disease patient advocacy organisations look like?

A strong collaboration between a patient advocacy organisation and a company should be of mutual benefit. The collaborations should start as early in the drug development process as possible and be consistent. The relationship should also be meaningful in nature and reflect a true partnership (4). That means that the pharma company should demonstrate a genuinely patient-orientated approach and that patient advocates should ensure regular and frank communication with the most important stakeholders – the patients.

In what areas should a company directly engage with patients and caregivers? Why are these important?

Companies should work with patients and caregivers in the areas of clinical trial design, patient-reported outcomes for trials, reviews of patient-facing resource materials, product packaging designs, comprehension of the patient journey and patient support programmes. One approach may be to involve the patient and caregivers, as well as other stakeholders involved in disease management, in an advisory capacity. An advisory board can provide meaningful input throughout all the stages of drug development and through regulatory approvals. These activities are important because they provide us with first-hand insight into what really matters to those affected by the disease.

Are there regulatory and compliance standards to be adhered to?

There are organisations that set regulatory and compliance standards for patient engagement. In Europe, the organisation is called the European Federation of Pharmaceutical Industries. A similar group in the US is called Pharmaceutical Research and Manufacturers of America. As Zambon is Italy-based, we also adhere to the standards set by Farmindustria. These organisations provide our industry with guidance around ethical standards and require total transparency in partnerships with patient organisations.

What background is needed for this role?

Ideally, an individual that is working in a patient advocacy role should have experience working with patients.

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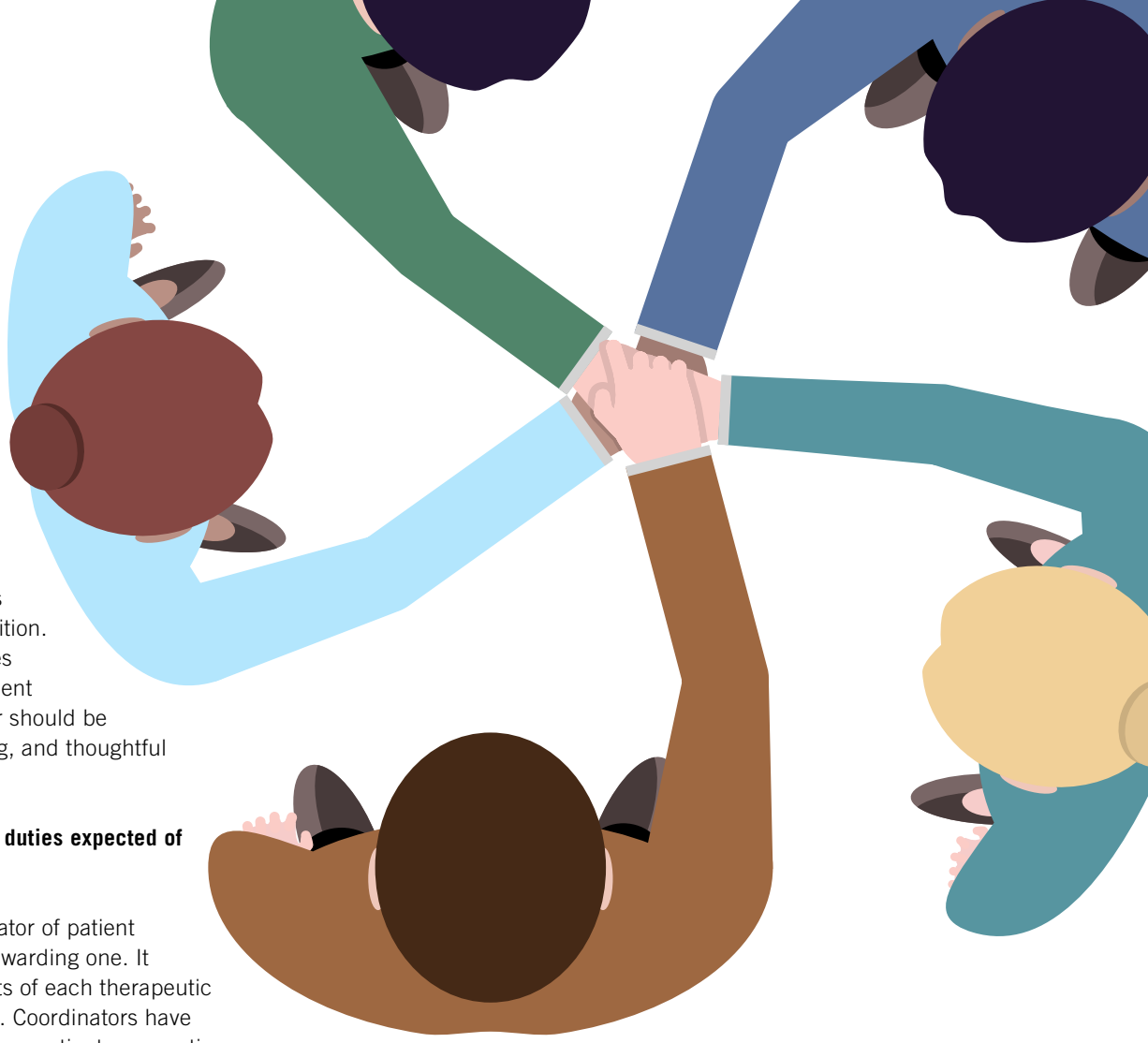

This background can be at a professional level, such as a healthcare practitioner who has cared for patients. It can also be from personal experience in caring for a loved one, such as a child or parent that suffers from a medical condition. While it probably goes without saying, a patient advocacy coordinator should be an empathetic, caring, and thoughtful individual.

What are the specific duties expected of a patient advocate?

The role of a coordinator of patient advocacy is a truly rewarding one. It involves many aspects of each therapeutic area and programme. Coordinators have the opportunity to share patient perspectives and provide input gained from their clinical experience in caring for patients. They're also responsible for all activities involving patient organisations and for direct communications with patients, when appropriate.

References

1. *Nguengang Wakap S* et al, *Estimating cumulative point prevalence of rare diseases: Analysis of the Orphanet database,* Eur J Hum Genet *28(2): pp165-73, 2020*
2. *Visit: www.bionj.org/wp-content/uploads/2015/04/BioNJ-White-Paper.pdf*
3. *Forsythe LP* et al, *A systematic review of approaches for engaging patients for research on rare diseases,* J Gen Intern Med *29 Suppl 3(Suppl 3): s788-800, 2014*
4. *Bloom D* et al, *The rules of engagement: CTTI recommendations for successful collaborations between sponsors and patient groups around clinical trials,* Ther Innov Regul Sci *52(2): pp206-13, 2018*

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Jennifer McGrain, MS RRT, is Coordinator of Patient Advocacy at **Zambon**. Zambon is an Italian family-owned multinational chemical and pharma company founded in 1906. Jen has 20 years of experience working in healthcare. Her early career was spent working at the bedsides of patients as a Respiratory Therapist. She then helped patients while working as a Clinical Research Coordinator in Pulmonary and Critical Care departments. Before moving into pharma, Jen worked at Hartford Hospital, the Pennsylvania State University's Hershey Medical Center, and the University of Maryland Medical Center, US. Jen's role in patient advocacy has been a natural transition of approach to impact patients in a positive way. She is responsible for incorporating the patient voice into all programmes at Zambon.