



Training on trials:

Patients taught the language of drug development

By Shraddha Chakradhar

While patient-centric approaches to research and development are gaining in popularity, another challenge is starting to present itself: how can patients engage with members of the industry in such a way that they work more as partners? This was a question that 29-year-old Vesna Stojmirova contended with while looking for a way to access treatment for her rare disease.

“Talking to pharmaceutical companies or government, you find yourself in a situation where you don’t understand what people are talking about, but you want to and need to understand,” says Stojmirova, a patient advocate based in Macedonia. “It’s important to have those conversations.”

Stojmirova was diagnosed with Gaucher disease at the age of seven, but getting access to the right treatment was a struggle. In 2009, she learned of a promising drug—Genzyme’s Cerezyme—but the cost was close to €200,000 (\$228,000) for a single year of treatment. Her doctor applied to have Stojmirova and one other person get the drug directly from Genzyme through the Cerezyme Emergency Treatment program, and luckily for them, they were chosen as recipients. Stojmirova continues to get the treatment today for free.

Now that her condition is stable, Stojmirova’s advocacy extends beyond her own rare disease, as she has also founded

an organization called Life with Challenges, which is dedicated to providing support to people with rare diseases as well as working as an advocacy organization on their behalf.

To better help with her role as a patient advocate, Stojmirova is now one of 49 participants in a new training course being offered by the European Patients’ Academy on Therapeutic Innovation (EUPATI), a consortium of 30 organizations from around Europe dedicated to providing information and resources on the medical research and development process to people affected by disease. The expert training course is designed to teach patients—current and former—or family members of patients all about the clinical trial process, from preclinical research and development all the way through the clinical trial and approval pipelines. The hope is that trainees will go on to become actively involved in the clinical research and development process—whether it’s by working with pharmaceutical companies or serving on regulatory agencies’ committees—or to serve as resources for other patients confronted with similar problems.

A need to engage

The director of EUPATI, Jan Geissler, is also a former patient. “I was diagnosed with chronic myeloid leukemia at the age of 28,

and, by coincidence I found a phase 2 trial,” says Geissler, a former media technology researcher. “I learned from other patients what was going on as far as research, but I soon learned that policy decisions are being taken on the European level [and] there is a need for more patient involvement.” The idea for both EUPATI and the course grew out of this realization that more patient engagement was needed. According to Geissler, who has advised on trial design and protocol, many people on the development side often fail to take into account real-world challenges when developing a clinical trial.

“I was involved in reviewing the protocol of a pediatric trial, one which included a bone marrow biopsy of children every three months [and giving the children] six tablets every day,” explains Geissler. “While it was scientifically interesting, I had to explain to the trial designers that parents would never enroll in such a trial.” The challenges of daily medication intake coupled with having to have the child undergo a painful procedure would deter recruitment.

“People are more interested in determining the efficacy of a drug, rather than taking into consideration quality of life,” says Geissler. Furthermore, especially when it comes to people like Stojmirova who suffer from rare diseases, “drugs are not really meeting the needs of patients.” Costs of medications are

often high and the side effects might be more unmanageable than the original symptoms. Geissler believes that involving patients while designing trials and developing drugs will help so that non-scientific factors that are still crucial to evaluating a drug's efficacy can be taken into consideration.

But for patients to be able to participate in the drug development process, they first need an initiation into the research and development world. The EUPATI course, which began in October 2014, is designed to be just such an initiation.

"If you want to challenge a process, you need to understand what the process is," says Geissler. "When patients don't understand the process, they might delay the process by suggesting things that may not be feasible. It might frustrate people on the development side."

The course will run for 15 months, at the end of which participants who have successfully completed its six modules will be awarded a certificate from EUPATI. The course is free of cost, operating like a scholarship program for the nearly 50 successful applicants from a pool of over 300 applications. Even though the course is free to participants, most of the operating costs for EUPATI are minimal because the training course is largely offered online.

Full course

The modules are challenging, according to both Stojmirova and Geissler. The participants have currently completed two



In training: EUPATI offers workshops to patient advocates.

modules—on the discovery of medicines and preclinical testing—and are currently in the third module, which covers exploratory and confirmatory clinical trials. All participants must pass an exam at the end of each module to proceed to the next one.

At the halfway point, after three modules, all the participants convene for a weeklong in-person training workshop in Barcelona, where they will have an opportunity to meet the other participants as well as discuss and analyze what they have learned during the course. Another such workshop will be held at the end of the course.

The course materials for the various modules are provided by various R&D experts, from academic researchers to ethicists and clinicians to industry representatives. "A lot of medicines are being developed by industry, so we need them to share their expertise with us," Geissler.

Nothing this comprehensive has been offered either by EUPATI or other organizations in Europe, according to Geissler. Most patient training is limited to day- or weekend-long workshops. In the US, organizations such as the Professional Patient Advocate Institute based in Gaithersburg, Maryland, offer certificate courses in patient advocacy whereas other organizations, such as the Alliance of Professional Health Advocates, provide resources for patients and patient representatives about workshops, formal training programs and conferences.

Making connections

For those like Stojmirova who are enrolled in the course, enrollment in the EUPATI course is largely about better understanding the clinical trial process as well as connecting with other patients, patient advocates and industry

representatives from across Europe. "Some of them are being invited by the European Medicines Agency to talk about R&D with people in the agency," says Geissler.

Here in the US, Ken Getz, founder of the Boston-based Center for Information and Study on Clinical Research Participation, says that private organizations are increasingly extending invitations to patient advocacy groups and patients where they can spend two to three days with members of the organization and learn about what they're doing as far as drug development and clinical testing are concerned. As one example, he points to the Drug Information Association, based in

Washington, DC, which serves as a forum through which professionals involved in the drug development process can engage with patients and patient advocates.

"In a time when the industry was performing well, there wasn't an incentive to do something new," adds Getz. "Now they really have to figure out new ways to do R&D." And those new ways might mean speaking with patients about their experiences being in clinical trials or having trouble accessing drugs. Getz is chairman of the Patients as Partners conference, an annual meeting that will be held in Philadelphia this month, which aims to bring together patient advocacy groups and industry representatives to facilitate larger involvement of patients in the clinical research arena.

Getz is skeptical of EUPATI's model for training. "I'm not sure that the best way to do this is through a course," he says, adding that there could be attrition through the long 15-month period. But he believes that patient engagement is still "fashionable" to industry, perhaps explaining why some but not all companies are starting to engage with patients.

Ultimately, the idea behind EUPATI is to empower patients with information that, in the end, affects their involvement in research, whether it's by participating in clinical trials or offering feedback to improve clinical trial design. "It's nice to have these kinds of training courses for advocates because patients are getting stronger," says Stojmirova. "We're building capacity for patient organizations, and this way we can have more power and more say in the [drug] development process."

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Course lead: Jan Geissler, the director of EUPATI.