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Three resonating patient-centric initiatives

Study volunteer advocacy training, clinical research concierge services and trial results summaries gaining momentum

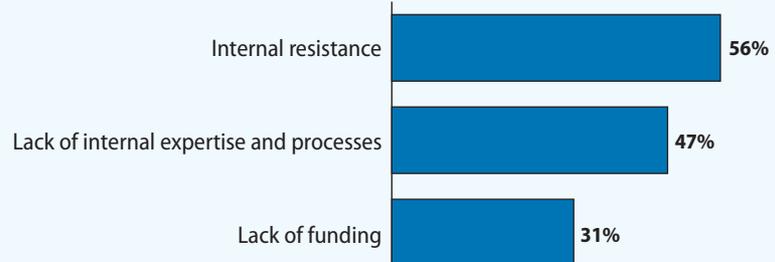
By Karyn Korieth

Sponsor companies are piloting patient-centric initiatives at a furious pace. Although few initiatives designed to engage patients as clinical research partners have achieved widespread adoption, several are making substantial inroads. In this issue, CenterWatch profiles three major patient-centric initiatives that appear to be resonating across the clinical research enterprise.

Insights from these profiled initiatives may guide clinical research professionals in anticipating sustainable patient engagement approaches that appear poised to generate public and patient community ownership in the success of clinical research programs.

Top perceived barriers to adopting patient-centric initiatives

(Percent of total responses; multiple choices accepted)



Source: CenterWatch 2014; (N= 68 sponsor and CRO companies)

Training patient advocates

The move toward a patient-centric drug development model has created the need for patient advocates who can effectively work as equal partners with drug developers and regulatory authorities throughout the drug R&D processes. Patient advocates don't always have a deep understanding of the various activities required to develop new medical treatments, yet they are increasingly asked for advice about complex issues such as protocol design, informed consent, marketing authorizations or health policies.

In response, the European Patients' Academy on Therapeutic Innovation (EUPATI), a public-private partnership led by the European Patients' Forum (EPF), was established in 2012 to provide training and educational resources designed to help patients and their advocates understand drug R&D and become more actively involved during each stage of the process, from preclinical research and development all the way through clinical trials and drug approvals.

"Patients have a unique perspective on

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More attractive study conduct market invites competition

Site operating margins increase and grant spending hits record levels

By CenterWatch editorial

Profit margins are up at investigative sites. This is welcome news as 2016 promises record levels of study grant spending mixed with intensifying competitive pressures in the study conduct market.

A recently completed CenterWatch online survey among 252 experienced investigative sites finds that profit margins have improved substantially. Average reported profit margins in 2015 at these investigative sites were up nearly four percentage points, at 13.9%. This compares with average reported profit margins of 10.1% in 2010. In open-ended responses to the global online survey, principal investigators and study coordinators indicate that they are making business-side improve-

ments to boost operating efficiency. Specific areas of improvement mentioned include budgeting, financial management and leaner staffing. Several investigators also note that more profitable studies—including vaccine trials—played a large role in their mix of active clinical trials in 2015.

Profit growth expectations are also high: 63% of investigative sites anticipate that their profitability will increase "strongly" (13%) or "somewhat" (50%) in

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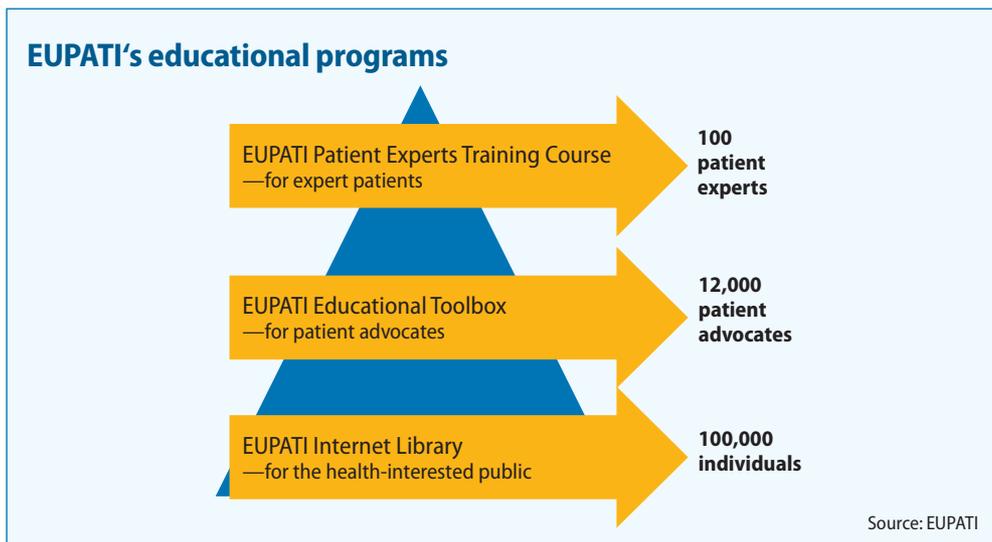
Patient-centric

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their needs and desires in terms of treatments, symptoms, side effects, quality of life and an adequate balance between the benefits and risks of treatment and research,” said EUPATI director Jan Geissler. “A number of studies, for example in CML (chronic myeloid leukemia) and myeloma, have evidenced that there is a significant difference in perceptions between healthcare professionals and patients in terms of severity of symptoms and side effects. Research and development might just end up with the wrong outcomes if patients and their unfiltered needs are not involved in research design.”

The EUPATI initiative, supported by more than 30 European organizations, offers an intensive 14-month training course created to help patient advocates develop the knowledge and skills needed to engage confidently with researchers, drug sponsors and other stakeholders at the European and national levels. Other resources include the EUPATI Toolbox, which includes articles, videos, presentations and fact sheets about clinical research that patient advocates can download in seven European languages, and a comprehensive library of online resources about drug R&D geared toward both patients and the lay public.

“Patients can become better advocates in our discussions with, for example, regulatory authorities and sponsors. If we are at the negotiation table, we need to speak their language. Otherwise, we are not an interesting party for them,” said Marleen M. Kaatee, founder and president of PSC Patients Europe, a foundation focused on finding a cure for a rare liver disease called Primary Sclerosing Cholangitis (PSC) and improving the quality of life for PSC patients, and a 2015 graduate of the first EUPATI expert patient training course.



“Patient advocates and ambassadors need to have the capacity and capability to understand how they can collaborate in medical research and development with all the other stakeholders.”

EUPATI doesn't provide information about disease-specific issues or therapies, but rather focuses on processes and issues

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—Marleen M. Kaatee, founder and president, PSC Patients Europe

related to drug R&D and the possible role of patient advocates and organizations in development of new medicines.

By the end of the year, about 100 patient advocates from 12 European countries are expected to have completed one of the two EUPATI expert patient training courses conducted since 2014. More than 200 patients and advocates applied for each session. The course, which is free for participants, requires students to complete six in-depth online modules and attend two

four-day in-person training workshops in Barcelona, where participants meet other patient advocates and discuss what they have learned during the course. Participants must pass an exam at the end of each module in order to progress to the next section; those who successfully complete the course become EUPATI Fellows, a designation that

is endorsed by the European Medicines Agency (EMA) and the Innovative Medicines Initiative (IMI).

EUPATI keeps a database of its patient experts and helps facilitate patient collaborations with academic and industry researchers, regulatory authorities and ethics committees. EUPATI-trained advocates have participated in patient involvement activities

across the drug R&D spectrum, including preclinical research, phase I-III clinical trials, regulatory approval and post-approval activities.

Kaatee believes patient involvement in the drug R&D process should begin as early as possible, beginning with discussing patient priorities in research with stakeholders and how disease affects their daily lives.

“We can be involved in the various phases of the clinical trial, preferably starting see [Patient-centric](#) on page 8

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with phase zero. When researchers want to start researching something, they can first check with us and see if it's really something that we want. We can work from there. Especially when you are dealing with a disease without a cure, like PSC, the patients might have another priority," said Kaatee.

As a EUPATI Fellow, Kaatee has assisted researchers with grant applications for PSC-related projects and advised industry and regulatory stakeholders about inclusion/exclusion criteria that could impact the willingness of PSC patients to participate in a clinical trial. For example, her foundation conducted a survey of PSC patients that found in some European countries, potential participants would not join a clinical trial if a liver biopsy was required. This is critical information when designing a global clinical trial for a rare disease. Kaatee has offered patient perspectives in meetings with Dutch health authorities, the Dutch Clinical Trials Foundation and pharmaceutical companies. Among many other efforts, she participated in European Medicines Agency patient-focused drug development workshops and worked with researchers and patient groups in Europe, the U.S. and Japan.

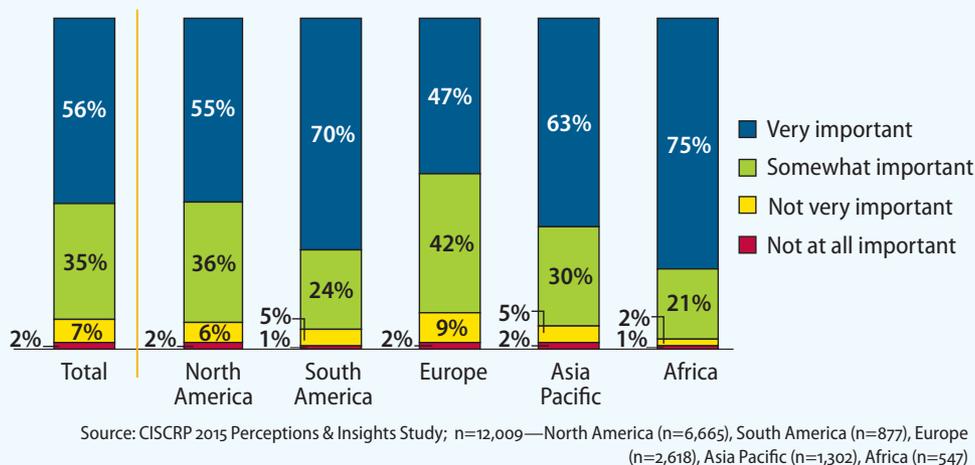
"I figured out very early on that if I wanted to be at that negotiation table, if I wanted to be an equal partner, I had to have the knowledge. What I see now is that I can really have impact," said Kaatee. "It's a great time to be a patient advocate because you can really make a change. But it's give and take. You need to take responsibility for being an educated patient."

As patient-centered drug development evolves, Kaatee believes researchers and drug sponsors need to develop a better understanding of how to incorporate patient perspectives into drug R&D and ways to work with patient advocates.

"The researchers also need to be trained as to how to incorporate the patient view

Perceived importance of plain language trial results summaries

How important is it to you to receive a summary on the results of the clinical research study?



and work with patients. There are huge differences among the different patient advocates about experience, training and willingness. For some stakeholders, it's still just, 'Let's invite the patients.' That is fine. But then they really see the possibilities. It has to grow," she said.

Easing burdens of participation

New approaches are emerging designed to take into account real-life patient needs and make study participation more convenient. Sponsors and CROs, for example, have used home nursing services and telemedicine to allow patients to participate from their own homes. Companies also are piloting electronic consent forms and wearable devices that can improve study volunteer experiences and make it easier to collect patient-reported data.

The Philadelphia-based Clincierge recently won a 2016 *Clinical Informatics News* best practices award in recognition for its service, which provides personalized support for study volunteers and their families. The service reduces the unexpected costs and burden of coordinating travel and overnight stays required for some patients to participate in a clinical trial. Employees are located in some 30 countries, called Clincierges or "clinical concierges," and also provide per-

sonal assistance to clinical trial participants, manage study visits and answer questions in the patient's own language.

Clincierge CEO Scott Gray said offering concierge-type services to study participants can lessen anxieties about trial-related travel and logistical issues that could deter patients from enrolling and completing studies. Ultimately, he said the company's goal is to reduce overall clinical testing time and costs by making clinical trials more patient-centric.

"For the most part, there is a lack of social interaction in how clinical trials are operated. It's not a hospitality-themed discussion between the site and the patient," said Gray. "For true patient centricity, there need to be methods of connecting with patients and removing as much anxiety about participation as possible."

Trial logistics, including the distance to the investigative site, the frequency of study visits and out-of-pocket costs for travel and meals, remain a significant barrier to patient participation in clinical trials. This is particularly true in rare disease studies where there are typically only a small number of sites in each country able to perform the complex study and patients must be recruited across multiple countries and wide geographic areas. In some cases, patients must travel to another country for study treatment where they don't speak the language.