Patients' voices carry: Drug development increasingly driven by patients' needs and concerns

July 23, 2015

This client alert is the second of a three-part series that discusses hot topics to come out of the 2015 BIO International Convention.

With the recent announcement of Sarepta's filing for approval of its drug, eteplirsen, for the treatment of Duchenne muscular dystrophy (DMD), coming on the heels of BioMarin's announcement a few months earlier of a similar filing, it has become clear that the role of patient advocates and patient-centered outcomes has had a significant favorable impact both upon the FDA and the development of products for rare diseases.

At the recent BIO annual meeting, one track focused specifically upon patient-centered efforts to shape regulatory and science policy. One panelist noted that efforts that began under the Bush Administration, such as insurance reforms valuing patient-centered outcomes, and continued in the Obama Administration, including the creation of the Patient-Centered Outcomes Research Institute (PCORI) by the 2010 Patient Protection and Affordable Care Act, have resulted in a far greater role for the voice of patients than has been seen to date.

Even while progress has been made, additional work continues. With the introduction of bipartisan 21st Century Cures legislation introduced by Energy Commerce Committee Chair Fred Upton (R-MI) and Democratic Deputy Whip Diana DeGette (D-CO), Congress is trying to further spur progress in the treatment of rare diseases and in addressing unmet medical needs. Among a number of important provisions originally included in the 21st Century Cures legislation was the Dormant Therapies Act. First introduced in 2011, this provision would assign "dormant therapy" status to a drug or new biological product that has been determined to have insufficient patent protection and meets an unmet medical need, improves outcomes, or reduces risks compared to an existing product. Several panelists at BIO noted that without this provision, the best patent—not the best drug—is often developed, leaving patients without access to the better products. The Dormant Therapies Act would try to address that. While the latest version of the Cures legislation does not include this provision, one panelist noted this was the top request of Alzheimer's groups on Capitol Hill during this current session.

In terms of the effectiveness of patient advocacy, several panelists pointed to the fact that the Parent Project Muscular Dystrophy (PPMD) was able to get formal DMD guidance through the FDA in early June, which was a major accomplishment. ALS patient advocacy groups are working for a similar outcome. One of the leading Alzheimer's patient advocacy groups, noted that FDA has granted Alzheimer's groups a chance to sit with the neurology review division, and has set up cross- center coordination. As a result, the posture of the FDA has changed, favorably, for Alzheimer's. This type of non-product-specific engagement with the FDA is likely to be more common as the Agency's positive reaction to these types of interactions becomes known to patients and patient advocates.

The panel noted that one of the recognized obstacles to greater patient involvement in early drug development is that researchers in rare diseases used to be clinicians—now far more researchers are laboratory-based PhDs. This has resulted in more disengagement with patients by drug developers, meaning a larger role of patient advocates is critical for the FDA to hear the voice of the patient. Similarly, it was noted that if biotech and pharmaceutical

companies are going to truly engage a patient advocate, the industry is going to need regulatory predictability on the role of patient-derived data during the FDA regulatory process or else there is no incentive to gather such data. Historically, companies tended to reach out to patient advocates at the last minute for support as a drug approached the finish line for approval. Today, patient advocates drive a lot of the agenda for the entire drug development process—not only for which drugs get approved, but for which drugs may be developed.

As Congress approaches reauthorization of the Prescription Drug User Fee Act (PDUFA) in 2016, the consensus coming out of BIO is that the goal of PDUFA 6 should be integrating the patient voice into drug development—and the Hill appears to get that. The panel at session Putting Patients in the Center: Advancing the Science of Patient Preference Assessment noted that there should be a "patient trifecta" of considerations as PDUFA 6 is contemplated by Congress, namely:

- What is the clinical outcome you are trying to achieve? Does a patient hope only for a cure or does he or she also value improvement of quality of life?
- What is going on in the patient's life? Can he or she access a clinical trial, many of which increasingly are taking place at centralized academic centers that are often far from rural areas? Can the patient get the care he or she will need?
- Why does the patient want to live? What are his or her short- and longer-term aspirations for undergoing care?

Drug companies and regulators have to look at the whole picture to decide which products to develop and approve, taking into account these core patient considerations. Patients are capable, reliable sources of what they want and need. Early and effective outreach, not only with patients and their advocates, but with the regulators who value patient input and the policymakers who look to affect change in the drug development process through legislation, is likely to further enhance a patient-centered drug development process and improve outcomes.

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