FDA Rare Disease Patient Advocacy Day Wrap-Up

On March 1, 2012, the Food and Drug Administration (FDA) convened its first "FDA Rare Disease Patient Advocacy Day" to give rare disease patient advocates the opportunity to meet with FDA staff and learn more about how FDA works. Experts gave informative presentations that were supplemented with interactive discussions in front of 216 patients, their families, industry and advocacy group representatives, with an additional 181 participants joining via live webcast.

Highlights from the day's agenda included a commentary from the FDA Commissioner, Dr. Margaret Hamburg who reinforced the FDA's dedication to rare diseases. The keynote address by Dr. Stephen P. Spielberg, FDA Deputy Commissioner for Medical Products and Tobacco, addressed the impressive progress made in the rare disease arena in the last few decades, including gene sequencing and the approval of a new targeted treatment for cystic fibrosis.

Dr. Gayatri R. Rao, Acting Director of the Office of Orphan Products Development, as well as each Center within the FDA, representing drugs, biologics and devices, presented their perspectives on products for rare diseases. This prompted participants to ask questions on what they could be doing to help researchers bring their discoveries to clinical trials and to prepare the rare disease community for participation. The ensuing discussions touched upon patient registries, natural history studies, and adverse event rates. Repurposing of approved drugs for new rare disease indications, for which the financial incentives of the Orphan Drug Act would still apply, was also mentioned.

The Office of Special Health Issues, which administers the Patient Representative Program and conducts training for patient representatives, outlined ways in which patients and advocates can get involved in the regulatory process. The Center for Drug Evaluation and Research Drug Shortage Program clarified the role of government, including its limitations, in responding to market shortages.

During lunch, on-site attendees had a unique opportunity to engage with FDA and outside experts in small group settings. In addition, breakout sessions provided a venue for more in-depth discussions regarding hot topics on rare diseases. A few of the topics discussed included risk tolerance in clinical trials, conflict of interest issues for patient representation on FDA advisory panels, and regulation of medical foods.

This meeting helped highlight our shared goal with the rare disease community by bringing new therapies to market as quickly as possible with adequate information about benefits and risks to guide decision making. It is hoped that participants came away from the meeting with an enhanced understanding of FDA's role in product development as a regulatory agency along with ideas on how patient advocates can become involved in the process.

We would like to acknowledge all of the speakers and co-sponsors, to include the National Institutes of Health, Genetic Alliance and National Organization for Rare Disorders, for their outstanding efforts in support and planning of this event.