DIA 2013 49th Annual Meeting
"Advancing Therapeutic Innovation and Regulatory Science"
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Boston, MA

Session 277 – Breakthrough Therapy: One Candle on the Birthday Cake – Are Innovators Enjoying Sweet success or Is the Pathway Not Baked Yet?

This was one of several DIA 2013 sessions on the FDA's breakthrough therapy rule highlighting how much of a stir it has really been making in the proverbial pot. Nancy Myers led the discussion with panelists who have really been in the trenches with their experience on this subject. Jeff Allen brought to the table firsthand knowledge of the legislature, Earl Dye brought in his filing experience from Genentech/Roche along with 14 years of wisdom from his time at the FDA, and lastly Urte Gayko, who is considered somewhat of a "breakthrough status champion" with the third filing for Pharmacyclics under her belt in the last year.

The goal of the breakthrough status designation is to expedite development and review of drugs for life threatening conditions that demonstrate substantial improvement over current standard of care. It allows the sponsors more face time with the FDA in addition to involvement of the senior staff and timely responses. Over 20 breakthrough status designations have been awarded since the inception of the rule and a large majority of those are in oncology with other notable disease areas being cystic fibrosis, heart failure and hepatitis C.

The panel collectively agreed that sponsors had good chances of success with their filing if they had robust Phase I data that showed significant improvements in end points compared to current standard of care (for single agents as well as combination therapies). However they did acknowledge that this flurry of filing successes came from a "queue" of drugs that were further along in development and were "sure success stories" that would have achieved breakthrough status if the rule existed at the time they were in Phase I trials.

The panel also cautioned the audience that while breakthrough status decidedly reduced time taken to take the product to market, it came with its own set of challenges. An accelerated timeframe from discovery to launch also meant that this period is extremely resource intensive and resource decisions that were made over a ten-year period now have to be made in five.