Session Report: Patient Advocacy in Product Development



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"The customer is always right" is a common expression followed by many top businesses. The customer has always been seen in the business world as the driving force and ultimate power behind decisions made and policies implemented. However,

in health care - particularly in medical product development the "customer" has just recently gained a voice in the crucial steps a product takes through the rigorous paths and channels of the FDA. The DIA 2012 Annual Meeting offering Patient Advocacy in Medical Product Development: The Evolving Relationship Between FDA & Its Patient Stakeholders (Session 312) analyzed the steps the FDA is taking to ensure that the patient is an integral aspect of this whole process.

Session chair James E. Valentine, MHS (Office of Special Health Issues, Office of the Commissioner, FDA) began by presenting the background and evolution of connecting the patient to the medical product development process. The OSHI was originally created in the late 1980's when HIV/AIDS patient protesters shut down the FDA for an entire day with protests so large that no one could move in or out of the building. These patients knew that HIV/AIDS medications were in the process of being reviewed for approval, but had no idea about the regulatory review process or how soon they might receive these medications. There was imminent demand for process transparency and for patients to play a role in making decisions about the drugs they would receive as therapy.

This started the FDA's patient representative program, which is still around today: The FDA recruits patients and patient advisors to serve on their review committees and has made the patient a key voice in their committee decisions. This patient program has amassed

tremendous results, such as the accelerated approval pathway in which patient advocates can voice their opinions and concerns.

As President and CEO of the International Cancer Advocacy Network, Marcia K. Horn has repeatedly worked with patient advocates and regulators. Her presentation continuously commended the FDA for their efforts to put patients first. As researchers come closer each day to finding a product to help cancer patients desperate for a cure, the voice of this patient population is more important than ever. The Agency has even implemented more easy to use paperwork such as single patient NDAs. All future plans point to an Agency that will continue to put patients first.

Patrick Frey (Office of Planning & Analysis, CDER) spoke mainly on the new benefit/risk assessment framework implemented by PDUFA V. This framework was initially developed by assessing previous cases that were labeled as difficult. The responsible review team was asked to identify and categorize how they determined an attribute was a risk or a benefit, and to write short benefit/ risk summaries that specifically addressed benefit/risk trade-offs that were made. This benefit/risk analysis framework tells the story of the specific problem, other current potential interventions, the benefit of the proposed intervention and specific concerns to monitor. A major policy change will allow patient information to be assessed earlier in the product development and review/approval paths.

The FDA takes patient advocacy very seriously and will publish a

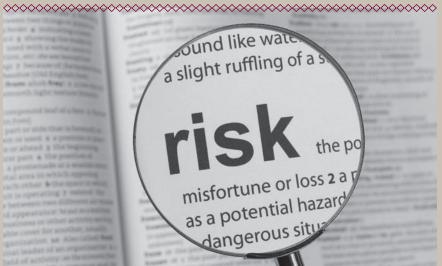
five-year plan to make their review and approval processes more transparent. This plan will include public workshops on twenty disease states open for public comment, one each quarter for five years, which will be invaluable in pinpointing unaddressed aspects of these diseases.

Mr. Marc Boutin, JD (COO of the National Health Council), provided insight regarding the changes that the government has taken to make drug development more patient-oriented. Mr. Boutin defined a "consumer" as an individual who uses the health care system for acute care, and a "patient" as an individual who will use the health care system regularly to manage their quality of life. This difference is crucial because what patients and consumers see as important in the benefit/risk relationship may not be the same. "Ten years from now, everyone will look back on PDUFA V and point to this as the most important part: That no other country has a qualitative framework for benefit versus risk," Mr. Boutin said. The passing of this legislation, along with its supporting policy changes and actions, will surely be significant for patients, healthcare professionals and regulators. These and other changes have created patient expectations that all government agencies will treat all disease states with equal importance.

As patients become more educated about the drug development process, FDA will continue to transform its culture to a state where the patient is just as important to new therapies as the scientist behind the molecules. As the Agency moves forward, a new

FDA Patient Network program will include a newsletter about recent and upcoming approval decisions, an annual meeting to address patient concerns, and a new

website that will serve patients looking to learn more about their illness plus patients who are more interested in advocacy.



Canadian Annual Meeting: Evolutions in Risk Thinking

DIA's focus on benefit/risk and its crucial role in drug development and lifecycle management will be discussed at our DIA Canadian **Annual Meeting: Evolutions** in Risk Thinking in Ottawa, Ontario. The meeting will open on November 5 with tutorials on How to Prepare a New Drug Submission and How to Prepare for a Pharmacovigilance Regulatory Inspection, then proceed through plenary sessions and three meeting tracks - drug development, pharmacovigilance and information technology/ communications - that will more comprehensively examine benefit/ risk topics on November 6-7.

Co-chaired by Neerja Goyal (GlaxoSmithKline, Inc., Canada)

and Paul Litowitz (Therapeutic Products Directorate, Health Canada), our DIA Canadian Annual Meeting is presented in collaboration with BIOTECanada, Canada's Research-Based Pharmaceutical Companies, the Canadian Association of Professional Regulatory Affairs, and the Clinical Research Association of Canada.

The opening plenary session on November 6 will explore *The Big Risks of Today; Forecasts for Tomorrow*, and will set the stage for more detailed conversations in subsequent meeting sessions. This plenary will feature Robert Cushman, MD, FRCPC (Director General, Biologics & Genetic Therapies Directorate, Health

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