NEW YORK PHARMA FORUM

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TOPIC: Leveraging Expanded Access Programs for New Drugs in Global Markets

SPEAKERS: **Jose Perez, MD**
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As patients and healthcare providers become more aware of pre-approval drug programs, such as expanded access and named patient programs, biopharma companies are increasingly receiving requests to make their investigational drugs available for treating serious conditions. In 2011 alone, 1,200 U.S. patients were treated through expanded access programs, a 20 percent increase over the previous year, for conditions such as hepatitis C, cancer and rare diseases like cystic fibrosis. Meanwhile, there has been greater interest in extending access to these programs in Japan and other countries. While there are many potential benefits for companies providing expanded access programs, there are also a number of challenges to consider, including the potential for negative PR and shareholder reaction in the event of negative clinical outcomes, the risk of compromising enrollment and drug supplies in ongoing clinical trials and the ability to recover the costs for drug manufacturing, just to name a few.

This program features a panel of industry experts who will share their experiences in managing expanded access programs across the globe, along with key insights on patient engagement from a leading patient advocacy group. Topics of discussion include:

- Overview of regulatory framework surrounding expanded access programs, including recent changes by the FDA and key considerations for managing such programs outside the U.S.
- Potential incentives and risks for sponsors of expanded access programs
- Strategies for effectively engaging key stakeholders, including patients, physicians and advocacy groups
- Forward-looking trends in expanded access and named patient programs in the U.S., EU, Japan and emerging markets