

Market Access and Reimbursement for Orphan Drugs and Rare Diseases

Joff Masukawa Interview

By Maeve McGovern

Citing the existence of over 6,000 rare diseases **Joff Masukawa**, Senior Director, Government Relations and Public Policy, Shire details his role in orphan product development and explains the importance of early engagement. Joff is a speaker at the **marcus evans Market Access and Reimbursement for Orphan Drugs and Rare Diseases Conference** taking place on November 16-17, 2010 in Philadelphia, PA.

Can you explain your role at Shire and how it relates to orphan product development?

JM: Since the birth of the Orphan Drug Act in 1983, the US government has provided incentives to companies for development of products for patients with rare diseases. These have helped to spur the commercialization of over 300 products to date. I work with advocates, government representatives and key stakeholders to ensure that policymakers understand the complexity of developing these products. There are over 6000 more rare diseases that have yet to be addressed, so much work remains for those with unmet needs.

Where do you see the orphan drugs market growing?

JM: As we learn more about genomics, scientists have more information about diseases and their patient populations. As this information and knowledge increases, improvements are being made in the ability to target new therapies for small patient populations. Such approaches will eclipse some of today's standards of care.

Do you see the Orphan Drug Act changing? If so, how?

JM: This is a question for Congress. It needs to assess whether incentives currently in the ODA are sufficient to incentivize the next generation of orphan drugs. The ODA was written more than 27 years ago. Many aspects of drug discovery and development have changed and pose more complex challenges now. The fact that FDA sought public comments earlier this year about the future of rare disease development is an acknowledgement of the changing landscape.

How do you work with various groups, like patient advocacy groups or CMS to ensure patient access?

JM: As early as the beginning stages of drug development, patient advocates can take an active and influential role in raising awareness of their disease and opportunities to participate in clinical trials. As research leads to product commercialization, advocacy groups can play a critical role in educating the reimbursement community. Early engagement with patient groups also helps drug companies better understand patients' needs and tailor programs to address them.

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