Rep. McCaul Seeks to Reinvigorate Interest in the Creating Hope Act of 2011; Introduces Companion Bill in the House

By Kurt R. Karst September 29, 2011

Last week, Representative Michael McCaul (R-TX), along with Reps. G.K. Butterfield (D-NC), Sue Myrick (R-NC), and Chris Van Hollen (D-MD), introduced H.R. 3059, the Creating Hope Act of 2011. The bill is a companion bill to S. 606, which was introduced in the Senate earlier this year by Senator Robert Casey (D-PA), along with co-sponsors Sens. Scott Brown (R-MA), Sherrod Brown (D-OH), Al Franken (D-MN), and Johnny Isakson (R-GA). Both the House and Senate versions of the Creating Hope Act of 2011, which are almost identical, are substantially similar to the 2010 version of the bill, S. 3697, which was introduced by now-retired Sen. Sam Brownback (R-KS). The Creating Hope Act would amend FDC Act § 524 to change the transferable Priority Review Voucher ("PRV") program created by the 2007 FDA Amendments Act (the so-called "treat and trade" program), and would amend the PRV program to extend it to applications for a "rare pediatric disease." Under the PRV program, sponsors of certain new drugs and biologics for "tropical diseases" that have received priority review may receive a PRV entitling the holder to a 6-month priority FDA review of another application that would otherwise be reviewed under FDA's standard 10-month review clock.

We previously reported on the Creating Hope Act of 2010 here, and we refer you to that post for a summary of the bill. The 2011 version of the bill, which we reported on here, makes some revisions to the 2010 version. The most significant change appears to be the conditions under which FDA may refuse to issue a PRV upon the approval of a rare pediatric disease product application – the so-called "good faith intent to market determination." Under both the 2010 and 2011 bills, FDA may consider several factors in determining whether to refuse to issue a PRV, including "the history of such sponsor of producing rare pediatric disease products for which such sponsor received a [PRV], orphan drugs for which the sponsor received exclusivity under [FDC Act § 527], or pediatric drugs for which the sponsor received an additional 6 months of exclusivity under [FDC act § 505A]." Added to the 2011 version of the bill is the requirement that FDA issue guidance before making a good faith intent to market determination.

Rep. McCaul, who co-chairs the House Childhood Cancer Caucus with Rep. Van Hollen, introduced H.R. 3059 on the day of the 2nd annual Childhood Cancer Summit. (The caucus website includes links to several stories on the Creating Hope Act and an endorsement video from actor Dennis Quaid.) In aDear Colleague Letter signed by Rep. McCaul and the bill co-sponsors, they urge support for the bill, stating that it "would expand and strengthen the cost-neutral FDA [PRV] program, giving pharmaceutical companies an incentive to develop treatments for rare diseases that are often less profitable than treatments for more common medical conditions." In addition, the letter notes that the possibility of a shortened review time offered by a PRV "is estimated to be worth hundreds of millions of dollars."

The true value of a PRV is debatable. BIO Ventures for Global Health hasestimated that, based on certain assumptions, a PRV could be worth a few hundred million dollars. The group cautions, however, that "[o]ne factor that may reduce the market value of priority review vouchers is risk tolerance. For example, companies may not be willing to invest as much in obtaining a priority review voucher if there are any doubts as to whether or not their product will be approved."

To date, FDA has issued only a single PRV – with the approval of Novartis' COARTEM (artemether; lumefantrine) for the treatment of acute, uncomplicated malaria infections in adults and children weighing at least five kilograms (see our previous post here). Novartis traded in the PRV (instead of selling it) to obtain priority review for a supplemental Biologics License Application for ILARIS (canakinumab) for the treatment of gouty arthritis attacks in certain patients, but an expedited review did not pan out for the company (see our previous post here). Thus, to date, the PRV market is untested. Adding further uncertainty to PRV value is the high PRV redemption user fee FDA has set. FDA recently set the Fiscal Year 2012 PRV redemption fee at $5,280,000 – and that amount is in addition to the Fiscal Year 2012 full application fee of $1,841,500.