Latest News & Updates from the Personalized Medicine Coalition

Letter from Washington

Today: A patient has late-stage non-small-cell lung cancer (NSCLC). She has gone through a number of treatments, but none were able to arrest the cancer's spread. The mother of four has a progressive disease and precious little time to waste on treatments that do not work. Her physician read reports of a newly approved drug, Xalkori® (crizotinib) that might offer hope. But only approximately 5% of NSCLC patients whose tumors have the anaplastic lymphoma kinase (*ALK*) gene rearrangement can potentially benefit. A newly approved diagnostic test determines whether or not the patient has the gene rearrangement, and therefore if the drug is a treatment option for her. After starting to take Xalkori, the tumors begin to respond.

So begins the forthcoming third edition of the Personalized Medicine Coalition's (PMC's) *The Case for Personalized Medicine*, a document that PMC will release on 16 November 2011 in Research Triangle Park (NC, USA) at a public event that considers the issues facing personalized medicine.

The Case for Personalized Medicine, the best introduction to the emerging field, defines personalized medicine for policy-makers, business leaders, journalists and healthcare professionals. Its goal is to increase awareness regarding personalized medicine, and in keeping with PMC's mission, catalyze investment in and accelerate the adoption of the new paradigm.

With the back-to-back approvals of two new paired personalized medicine products by the US FDA in August 2011, a drug and a diagnostic for late-stage NSCLC and another to treat patients with late-stage melanoma, our task is now much easier. We are reminded of the wonderful song from *My Fair Lady* in which Eliza exclaims, "Sing me no song/read me no rhyme/don't waste my time ... show me."

Although there are multiple examples of personalized medicine improving patients' lives (see Table 1 in *The Case for Personalized Medicine*), the addition of Xalkori (crizotinib) and Zelboraf™ (vemurafenib) to the growing list of personalized medicine drugs, treatments and diagnostics allows us to show more progress in the field.

Xalkori, approved together with a first-of-a-kind companion diagnostic test called Vysis ALK Break Apart FISH Probe Kit (Abbott Molecular, IL, USA), will allow physicians to treat a very small subset of NSCLC patients, not more than 6000 patients in the USA, who are most likely to respond to the drug. It works by blocking proteins produced by an abnormal *ALK* gene, and represents, according to the FDA, the 'trend in oncology research'.

Developed by Pfizer in a coordinated program with Abbott Molecular, which did the work on the companion diagnostic, Xalkori came to the market far in advance of the decade-plus average time it takes to develop a new drug. It was also approved ahead of the FDA's expedited 6-month review of drugs that offer significant improvement for patients, signaling that the FDA is serious about personalized medicine. Although the manufacturer will charge more than US\$100,000 a year per patient for the drug, its efficacy rates, at least twice most other cancer drugs, extended life for up to 48 weeks in clinical trials. The diagnostic test will retail for approximately \$250.

It is hard to imagine a drug such as this being developed 5 years ago. At least in oncology, we are rapidly moving into a new era of drug discovery and development.

Another example of progress was the approval, 9 days earlier, of Zelboraf (vemurafenib), also paired with a companion diagnostic; it will treat patients with melanoma that cannot be removed by surgery. That drug is especially indicated for treatment of patients with melanoma whose tumors express a gene mutation called *BRAF*V600E, which the drug blocks. Like Xalkori, the FDA approved it ahead of schedule.

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Although only 5000 patients a year in the USA are diagnosed with the *BRAF* V600E mutation, Roche, the drug’s manufacturer, estimates that sales could top \$1 billion a year based on a \$56,400 course of treatment for 6 months.

While the pharmaceutical industry may have been slow to recognize the value proposition in personalized medicine, it has now recognized that targeted therapeutics can help secure a drug’s approval, clear a path to reimbursement and lay the groundwork for a re-energized industry.

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