IN brief

Exelixis debuts in rare cancer

Exelixis launched its first drug, the oral tyrosine kinase inhibitor Cometriq (cabozantinib), for treating patients with metastatic medullary thyroid cancer (MTC), after US Food and Drug Administration approval on November 29. The agency did not require a risk evaluation and mitigation strategy for Cometriq, in contrast with London-based AstraZeneca's rival kinase inhibitor Caprelsa (vandetanib), the only other drug approved to treat the rare disease. In a phase 3 study, Cometriq extended progression-free survival for patients to 11.2 months compared with 4 months on placebo. The S. San Francisco, California-based biotech estimates an eligible patient population of 500–700 per year in the US, resulting in a modest marketing opportunity for Cometriq, whose monthly price is \$9,900. Exelixis is also studying Cometriq in prostate cancer, a much larger but also crowded market. The two phase 3 trials in prostate cancer, whose data are not expected before 2014, are testing the drug in late-stage, castration-resistant patients who have already progressed on other therapies. Eric Schmidt, an analyst at Cowen and Co., estimates that if Cometriq is able to demonstrate a meaningful survival benefit in prostate cancer, it could garner peak global sales in this indication of \$300–500 million.

Malini Guha

First drug for short-bowel

The US Food and Drug Administration (FDA) on December 21 approved Gattex (teduglutide), a first-in-class, ultra-orphan therapy for treating adults with short-bowel syndrome (SBS). SBS is a serious disorder in which the body is unable to absorb enough nutrients and fluids through the gastrointestinal tract and so patients must receive them intravenously. In phase 3 studies, Gattex given as daily subcutaneous injections improved intestinal absorption of fluids and nutrients, reducing—and sometimes eliminating—the need for parenteral nutrition. It is a recombinant analog of human glucagon-like peptide 2 (GLP-2), a 33-amino-acid peptide secreted in the gut to enhance absorption, increase vascularization and slow gastric motility. Pharmacological activities of Gattex are limited to the gastrointestinal tract, where most of the GLP-2 receptors are located. Despite a \$295,000 annual price tag, 200–300 patients are expected to try Gattex this year and peak sales are anticipated to exceed \$350 million, according to Francios Nader, CEO of NPS Pharmaceuticals in Bedminster, New Jersey, which is launching Gattex in the US this quarter. Gattex has been associated with cancer and other serious conditions, so the FDA has approved the drug with a risk evaluation and mitigation strategy. Last year, on August 30, the EU approved Takeda's drug Revestive (teduglutide) for SBS, but the Tokyo-based pharma has not yet launched it in the EU.

Emma Dorey

Startups, inventors cheer European unified patent

The EU has reached political agreement for a unified patent system, more than 40 years after the idea was first mooted. In December, the European Parliament voted positively on proposals to establish a unitary patent for Europe, promising an end to the current expensive and Byzantine system that small biotechs must navigate to fully protect their intellectual property (IP).

As the US transitions to a new IP system this month (**Box 1**), the EU says it expects to introduce its unified strategy in January 2014. The single EU patent will incorporate three new elements: a new system for registering and maintaining patents; a new language regime; and a single European court for dealing with disputes. The most immediate advantage will be cost savings. To have a patent recognized in the 25 countries signing up to the scheme will fall from an average €36,000 (\$48,000) to €4,725 (\$6,243) according to the European Commission. At the same time, having a single court will make it far less costly for inventors to defend their IP than in the current system, under which it

is necessary to file separate cases in different national courts.

At present, a patent granted by the European Patent Office (EPO) in Munich must be translated into the language of each country in which it is to be validated and then registered at the respective national patent office. In future, patents written in English, French or German will automatically be valid in each of the 25 countries that are signing up to be part of the new scheme. Translation costs, which Nick Wallin, partner and patent attorney at the London law firm Withers & Rogers, says are €8,000–10,000 (\$10,600–13,200) per language for a 100-page patent, will drop as will the cost of litigation. “The cost of enforcement is a huge issue; if you reduce that cost and make it easier for everyone, the value of your patents goes up,” adds Wallin.

In addition to granting patents, EPO will be responsible for levying annual renewal fees and distributing them to national patent offices. What rate EPO will be charging for granting and renewing unitary patents is one

Box 1 Twitter and Facebook prior art under new US patent system

Some US patent attorneys and inventors are keenly anticipating—some say, dreading—a key provision in the American Invents Act that takes effect on March 16. President Barack Obama signed the patent reform legislation in September 2011 (*Nat. Biotechnol.* **29**, 953–954, 2011), which shifts the US system from a ‘first to invent’ to a ‘first inventor to file’ system and thus aligns it with most other patent systems. The new provision was deliberately put on an 18-month hold to give officials at the US Patent and Trademark Office time to develop new rules, and for patent attorneys and their clients to prepare for a new, vastly different way of conducting business.

Expect a rush to file applications before the March deadline, patent experts say, followed by perhaps 10 to 20 years of confusion and litigation as inventors and their attorneys grapple with sharp differences between the old and new ways of evaluating those applications. Particularly confusing is that applications filed before and after the deadline will be subject to two very different eligibility regimens that could be subject to dispute for the 20-year lifetimes of their respective patents. These discrepancies are expected to cause considerable confusion as lawsuits and challenges come before juries and judges, who might find it difficult to keep those detailed differences in mind as they deliberate.

Perhaps the most important looming change is in the standard for defining prior art, when evaluating whether research described in an application is novel and otherwise worthy of patent protection. After the March deadline, prior art cited to disqualify an application will be very broadly defined and may include research reports or patents in obscure languages, public events in any country, disclosures on websites, and possibly even claims and descriptions posted on Facebook and Twitter. Inventors in biotech, who may be accustomed to free-flowing discussions during scientific conferences, may find themselves subjected to anxious coaching from patent attorneys about just what can be said during such discussions or disclosed in posters and papers.

Jeffrey L Fox Washington, DC