NEWS IN BRIEF

FDA approvals for the first 6 months of 2015

The US Food and Drug Administration (FDA) approved 14 new drugs in the first 6 months of this year (TABLE 1). These Center for Drug Evaluation and Research (CDER) approvals comprised 11 approvals for small molecules and 3 for biologics.

FDA approvals tend to be back-loaded to the second half of the year. Last year, the FDA approved 17 new drugs in the first half of the year and 24 in the second half, for a total of 41 approvals (*Nat. Rev. Drug Discov.* **14**, 77–81; 2015).

Separately, the FDA also approved its first biosimilar drug this year: Sandoz's filgrastim-sndz, a biosimilar of Amgen's filgrastim.

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Table 1 | **FDA approvals in the first 6 months of 2015**

Drug (trade name)	Lead company	Indication
Edoxaban tosylate (Savaysa)	Daiichi Sankyo	Stroke and systemic embolism in patients with non-valvular atrial fibrillation
Secukinumab (Cosentyx)*	Novartis	Plaque psoriasis
Parathyroid hormone (Natpara)*	NPS Pharmaceuticals	Control of hypocalcaemia in hypoparathyroidism
Palbociclib (Ibrance)	Pfizer	ER-positive, HER2-negative breast cancer
Lenvatinib (Lenvima)	Eisai	Thyroid cancer
Panobinostat (Farydak)	Novartis	Multiple myeloma
Avibactam plus ceftazidime (Avycaz)	Allergan	Complicated intra-abdominal infections and complicated urinary tract infections
Isavuconazonium (Cresemba)	Astellas Pharma	Fungal infections
Dinutuximab (Unituxin)*	United Therapeutics	Neuroblastoma
Cholic acid (Cholbam)	Retrophin	Bile acid synthesis disorders and peroxisomal disorders
Ivabradine (Corlanor)	Amgen	Chronic heart failure
Deoxycholic acid (Kybella)	Kythera	Fat below the chin
Eluxadoline (Viberzi)	Actavis	Irritable bowel syndrome
Cangrelor (Kengreal)	The Medicines Company	Myocardial infarction, repeat coronary revascularization and stent thrombosis

*Biologics. ER, oestrogen receptor; HER2, human epidermal growth factor receptor 2.

Vertex combo scores a broader cystic fibrosis approval

Vertex broke new ground in 2012 when regulators approved its ivacaftor as the first disease-modifying cystic fibrosis drug. But this initial approval was only for use in the ~4% of cystic-fibrosis patients that have G551D mutations in the gene encoding the cystic fibrosis transmembrane conductance regulator (CFTR) ion channel. With the US Food and Drug Administration (FDA)'s approval of Vertex's combination of ivacaftor plus lumacaftor in July, the treatment-eligible population expands to include patients with the most common *CFTR* mutation, F508del, which accounts for close to 50% of disease cases. For now, the combination is only approved in patients aged 12 years and older, but ongoing trials in patients as young as 6 could further expand its utility.

By combining lumacaftor, which stabilizes CFTR during the folding process, with ivacaftor, which potentiates the activity of CFTR at the cell surface, Vertex has succeeded in restoring some CFTR function. In pivotal trials, combination treatment improved measures of forced expiratory volume in 1 second by about 3% over placebo at 24 weeks. Before the approval, there were some concerns that this effect was too modest (*Nat. Rev. Drug Discov.* **13**, 713–714; 2014). But in May an independent panel of FDA advisors voted 12 to 1 in favour of approving the combination therapy.

Analysts are forecasting peak global annual sales of the combination of US$3.8 billion by 2019, according to a consensus sales estimate from Thomson Reuters Cortellis.

Vertex's VX-661, a follow-on to lumacaftor that also stabilizes CFTR during its folding, is in Phase III trials. Several other cystic fibrosis candidates, including a gene therapy, are also in development (*Nat. Rev. Drug Discov.* **13**, 721–722; 2014).

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Calculating cancer drug value

With the cancer drug market recently having hit US$100 billion, and some industry experts concerned that cancer drug prices are unsustainable, two groups have started working towards calculating the values of cancer drugs.

First, the American Society of Clinical Oncology (ASCO)'s Value in Cancer Care Task Force proposed a conceptual framework under which ASCO working groups will assess cancer drug value on a scale of up to 130 points (*J. Clin. Oncol.* 22 Jun 2015 [epub ahead of print]). Clinical benefit will be scored on the basis of improvements in median overall survival or progression-free survival to provide a maximum score of up to 80. A drug's toxicity profile can add or subtract up to 20 points. Up to 30 bonus points can also be added for relief from cancer-related symptoms or for drugs that offer improvements in treatment-free intervals. The authors propose that the calculated drug scores could eventually be presented alongside drug cost details "to assist the physician and patient in shared decision making as they work toward defining value and identifying an appropriate intervention for that individual patient".

Separately, Peter Bach, at Memorial Sloan Kettering Cancer Center in New York, USA, and colleagues launched an interactive calculator called the DrugAbacus to explore the value of 54 approved cancer drugs. The tool embraces the different possible underlying assumptions that can be used to value drugs. DrugAbacus users set a baseline value in terms of 'dollars per life-year provided' for all drugs, and then select toxicity discounts and 'novelty', 'cost of development', 'rarity' and 'burden of disease' premiums. DrugAbacus then calculates 'value-based prices' and compares these to the actual market cost of drugs.

When the value of a year of life is set at US$120,000 and the toxicity discount is set at 15%, only 9 out of 54 drugs (16%) are currently priced lower than their 'value-based price'.

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