Drug pipeline: Q210

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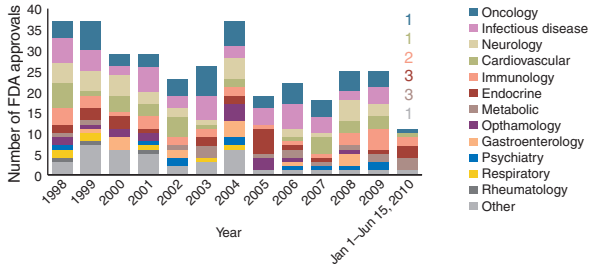
New drug approvals were off to a slow start in 2010 but addressed indications outside the usual areas. In April, the first autologous cell therapy, Provenge (sipuleucel-T), was approved, and last month, Amgen's RANK ligand antagonist (denosumab) was also registered for marketing. Fingolimod, the first synthetic sphingosine-1-phosphate agonist in multiple sclerosis, was given a favorable recommendation, and positive trial data came in for the antisense drug, mipomersen, as well as ipilimumab, epratuzumab and pertuzumab, which addresses a new epitope on HER2.

FDA approvals by therapeutic indication

Oncology, infectious, neurological and cardiovascular diseases have been absent from drug approvals this year



Source: U.S. Food and Drug Administration Center for Drug Evaluation and Research (FDA CDER); BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com/).

Notable regulatory approvals (March–June 2010)

Drug name	Indication	Company	Approval
Prolia (denosumab)	Post-menopausal osteoporosis	Amgen	FDA, 6/1/10; EMA, 5/28/10
Provenge (sipuleucel-T)	Prostate cancer, castration-resistant	Dendreon	FDA, 4/29/10
Menveo (MenACWY-CRM vaccine)	Meningococcal disease prevention for adults age 11–55	Novartis	FDA, 2/22/10; EMA, 3/18/10
Lumizyme (alglucosidase alfa)	Pompe disease	Genzyme	FDA, 5/25/10 (sBLA)

Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com/). sBLA, supplemental Biologic License Application; FDA, US Food and Drug Administration; EMA, European Medicines Agency.

Notable regulatory setbacks (Mar–Jun 2010)

Drug name	Indication	Company	Setback summary
Naproxcinod (nitronaproxen)	Pain, arthritis	NicOx	5/12/10 FDA advisory panel meeting voted 16 to 1 against approval. In phase 3 trial, naproxcinod treatment was superior to placebo (primary endpoint) but failed to achieve statistical noninferiority compared with secondary endpoint naproxen (Aleve) (*Osteoarthritis and Cartilage* **18**, 629–639, 2010).
Belatacept (LEA29Y)	Kidney transplantation rejection	Bristol-Myers Squibb	5/1/10 FDA complete response letter requested 36-month data from the ongoing phase 3 study. The initial BLA filing included only 24-month data.
Albinterferon alfa-2b (Zalbin, a.k.a. Albuferon or Joulferon)	Hepatitis C	Human Genome Sciences/ Novartis	4/19/10 marketing authorization application (MAA) withdrawal due to unfavorable EMA opinion. FDA issued unfavorable discipline review letter on 6/14/10.
Cerepro (sitimagen ceradenovec)	Malignant glioma	Ark Therapeutics	3/9/10 MAA withdrawal due to unfavorable recommendation from EMA advisory panel, following MAA resubmission in 02/10. FDA response to BLA expected in 06/10.

Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com/). BLA, biologic license application.

Notable trial results (Mar–Jun 2010)

Company/ drug name	Indication	Result summary
Bristol-Myers Squibb/ Ipilimumab	Metastatic melanoma	Phase 3 study showed monotherapy or combination with gp100 peptide vaccine significantly prolonged overall survival (primary endpoint) from 6.4 months to 10 months (*New Engl. J. Med.*, published online, doi:10.1056/NEJMoa1003466, 5 June 2010).
Genzyme–Isis Pharmaceuticals/ Mipomersen, s.c. (ISIS-301012)	Homozygous familial hypercholesterolemia	Phase 3 study met primary endpoint (low-density lipoprotein (LDL) cholesterol concentration decrease in treatment versus placebo; $P < 0.003$) as well as secondary and tertiary endpoints (*Lancet* **375**, 998–1006, 2010).
Tolerx–GlaxoSmithKline/ Otelixizumab (ChAglyCD3)	Diabetes mellitus, type I	Although primary endpoint (suppression of rise in daily insulin requirement) was not met in all subgroups, phase 3 study showed efficacy over 48 months, depending on patient's age and initial beta cell function (*Diabetologia* **53**, 614–623, 2010).
UCB–Immunomedics/ Epratuzumab	Systemic lupus erythematosus (SLE)	Phase 2b study showed clinically meaningful improvements in patients with moderate to severe SLE (Abstract for 2010 Annual Congress of the European League Against Rheumatism, 16 June 2010).
Vical/ Velimogene aliplasmid (Allovectin-7)	Metastatic melanoma	High-dose therapy well tolerated in single-arm, open-label phase 2 study, with 11.8% response rate among 127 patients. *Melanoma Res.* **20**, 218–226, 2010).
MolMed NGR-hTNF (Arenegyr)	Mesothelioma	Phase 2 study met primary endpoint and showed overall 46% patients achieved disease control with median progression-free survival increased from 2.8 month to 4.7 months (*J. Clin. Oncol.*, published online, doi:10.1200/JCO.2009.27.3649, 20 April 2010).
Roche–Genentech/ Pertuzumab (2C4)	Breast cancer, HER2 positive	Single-arm phase 2b study in conjunction with trastuzumab showed combination is active and well tolerated in patients with metastatic HER2+ breast cancer and responsive to previous Herceptin treatment (*J. Clin. Oncol.* **28**, 1138–1144, 2010).

Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com/).

Notable upcoming approvals Q310

Company/ drug name	Indication	Expected approval
Theratechnologies/ Tesamorelin (Egrifta/ ThGRF/somatorelin)	HIV-associated lipodystrophy	7/27/10 PDUFA date. FDA panel voted 16 to 0 in favor of approval on 5/27/10. Phase 3 study showed treatment met primary endpoint (*J. AIDS* **53**, 311–322, 2010).
Savient Pharmaceuticals/ Krystexxa (pegloticase)	Gout	9/14/10 PDUFA date. Biologic license application resubmitted in 03/10 to correct deficiencies cited by FDA in 08/09 following favorable panel vote (14 to 1) on 6/16/09.
Novartis/ Gilenia (fingolimod)	Multiple sclerosis	9/21/10 PDUFA date. FDA advisory panel voted in favor of approval on 6/10/10. Phase 3 study met primary endpoint. (Abstract in *Amer. Acad. Neurol.*, 15 April 2010).
Roche–Genentech/ Lucentis (ranibizumab)	Diabetic macular edema; retinal vein occlusion	H2 2010 supplemental MAA approval.

Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com/). PDUFA, Prescription Drug User Fee Act. MAA, marketing authorization application.

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