Drug pipeline: 1Q14

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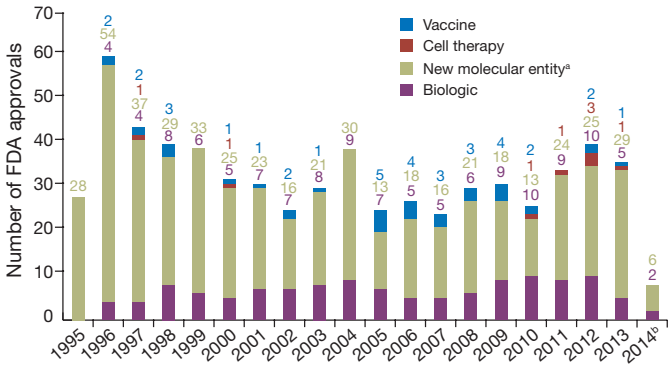
The number of drug approvals was down by more than half from 1Q13. Registrations reflected the march of biobetter drugs (Tanzeum; Alprolix). A first-in-class phosphodiesterase E inhibitor was approved for psoriatic arthritis. The cardiology field received a huge setback, with negative opinions for Novartis' highly anticipated peptide relaxin (Reasanz) in acute heart failure. PDUFA dates are approaching for Gilead's idelalisib against phosphoinositol trisphosphate kinase and Mannkind's oral insulin.

Historic US regulatory approvals by drug class

Approvals are down year-on-year.



[a]New molecular entity (NME) class includes mainly small-molecule drugs, but also steroid, synthetic peptide and mixed compounds. [b]Partial year to March 29. Source: US Food and Drug Administration, excluding non-NME drugs or new formulation.

Notable regulatory approvals (Q1 2014)

Drug/company	Indication	Drug information
Hetlioz (tasimelteon)/Vanda	Non–24-hour disorder	1/31/2014. FDA. Melatonin agonist to regulate sleep-wake patterns
Vimizim (rh acetylgalactosamine-6-sulfate sulfatase)/BioMarin	Mucopolysaccharidosis IV	2/14/2014. FDA. Recombinant enzyme replacement, the first drug to receive the FDA's Rare Pediatric Disease Priority Review Voucher
Myalept (metreleptin)/Amylin	Lipodystrophy	2/24/2014. FDA. rmet-HuLeptin to treat complications of leptin deficiency
Otezla (apremilast)/Celgene	Psoriatic arthritis	3/21/2014. FDA. Oral PDE4 inhibitor
Tanzeum (albiglutide)/ GlaxoSmithKline	Diabetes mellitus type II	3/26/2014. EMA. Long-acting glucagon-like peptide, formed by fusing rh albumin to GLP-1
Alprolix/Biogen IDEC	Hemophilia B	3/28/2014. FDA. Long-acting rFactor IX formed by fusing the Fc portion of IgG to Factor IX

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

Notable regulatory setbacks (Q1 2014)

Drug/company	Indication	Drug information
ABT-126/AbbVie	Alzheimer's disease	1/15/2014. Company suspending trial due to insufficient cognitive improvement
Ataluren/PTC Therapeutics	Muscular dystrophy	1/23/2014. EMA gave a negative review for exon-skipping molecule because of no improvement over placebo
Onartuzumab/Roche	Non-small-cell lung carcinoma	3/2/2014. Company suspended phase 2 trial of mAb against c-MET after missing endpoint
Imetelstat/Geron	Myelofibrosis, multiple myeloma, essential thrombocythemia	3/12/2014. FDA put a clinical hold on all trials with oligonucleotide inhibitor of telomerase because of possible hepatotoxicity
Reasanz/Novartis	Acute decompensated heart failure	3/27/2014. FDA and EMA issued negative opinions because of lack of efficacy
Bapineuzumab/ Johnson & Johnson	Alzheimer's disease	1/23/2014. Company suspended phase 3 trials of antibody against beta-amyloid after no benefit in patients with mild to moderate disease

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

Notable upcoming regulatory decisions (Q3 2014)

Drug/company	Indication	Upcoming catalyst
Afrezza/Mannkind	Diabetes mellitus, type II	7/15/2014. FDA PDUFA for this insulin system that has a dry formulation of insulin delivered with an inhaler
Suvorexant/Merck	Insomnia	8/1/2014. FDA PDUFA. Dual orexin A and B receptor antagonist
Idelalisib/Gilead	Chronic lymphocytic leukemia, small-cell lymphocytic lymphoma	8/6/2014. FDA PDUFA for this breakthrough first-in-class drug inhibitor of PI3 kinase
Plegridy/Biogen IDEC	Multiple sclerosis	8/21/2014. FDA PDUFA for this pegylated interferon-beta1a
Balugrastim/Teva	Neutropenia/ leukopenia	7/31/2014. EMA will review this Neulasta biosimilar (G-CSF albumin fusion)
Cobicistat with Prezista/Johnson & Johnson	HIV/AIDS	7/1/2014. EMA will consider this combination of HIV protease inhibitor with CYP3A inhibitor, which acts as a pharmacokinetic enhancer when used with other drugs
Secukinumab/ Novartis	Psoriasis	7/1/2014. EMA will consider this IL-17 inhibitor
Ch14.18 Mab/United Therapeutics	Neuroendocrine tumors	9/1/2014. EMA will consider this disialganglioside inhibitor

FDA, US Food and Drug Administration; PDUFA, Prescription Drug User Fee Act; EMA, European Medicines Act; IL, interleukin. Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com/).

Notable clinical trial results (Q1 2014)

Drug/company	Indication	Summary
AAV.REP1/ Nightstarx	Choroideremia and other blindness indications	1/16/2014. Phase 1/2 results for gene therapy with REP escort protein-1 showed improved rods and cones. *Lancet* **383**, 1129–1137 (2014)
Romosozumab/ Amgen	Osteoporosis/osteo-penia	1/1/2014. Phase 2 results for mAb against sclerostin showed treated patients reported less pain than controls. *NEJM* **370,** 412–420 (2014)
Solanezumab/ Eli Lilly	Alzheimer's disease	1/23/2013. Phase 3 trial showed no improvement with mAb against amyloid-β in phase 3 trial. *NEJM* **370**, 311–321 (2014)
Evolocumab/ Amgen	Dyslipidemia/hyper-cholesterolemia	3/21/2014. Phase 2 results for mAb against proprotein convertase subtilisin *Circ J.* http://dx.doi.org/10.1253/circj.CJ-14-0130 (21 March 2014)
ProHema/Fate Therapeutics	Bone marrow transplant/stem cell transplant	1/17/2014. Phase 1 study of umbilical cord stem cells showed treatment with prostaglandin improved survival of transplanted T cells. *Blood Cancer J.* **4**, e178 (2014)
SB-728/ Sangamo	HIV/AIDS	3/5/2012. Phase 1 study of autologous T cells with CCR5 receptor knock-out showed patients had decreased viral load. *NEJM* **370**, 901–910 (2014)
Human spinal cord stem cells/ Neuralstem	Amyotrophic lateral sclerosis	3/7/2014. Phase 1 study of spinal cord cells showed that cells can be safely transplanted. *Ann. Neurol.* **75**, 363–373 (2014)
REG1/Regado	Percutaneous coro-nary interventions	1/29/2014. Phase 2b study of oligonucleotide aptamer allowed early arterial sheath removal. *J. Invasive Cardiol.* **25**, 593–599 (2013)
EMA401/ Spinifex	Post-herpetic neu-ralgia	2/5/2014. Oral angiotensin type 2 receptor agonist pro-vided pain relief over placebo. *The Lancet* doi:10.1016/S0140-6736(13)62337-5 (5 February 2014)
Sofosbuvir + ledipasvir/ Gilead	Hepatitis C	2/8/2014. Phase 2 trial showed fixed dose of combina-tion with or without ribavarin can cure most patients. *The Lancet* **383**, 515–523 (2014)
Vacc-4x/Bionor Pharma	HIV/AIDS	2/11/2014. Phase 2 trial showed peptide vaccine was safe and effective at 48 weeks. *The Lancet* **14**, 291–300 (2014)

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

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