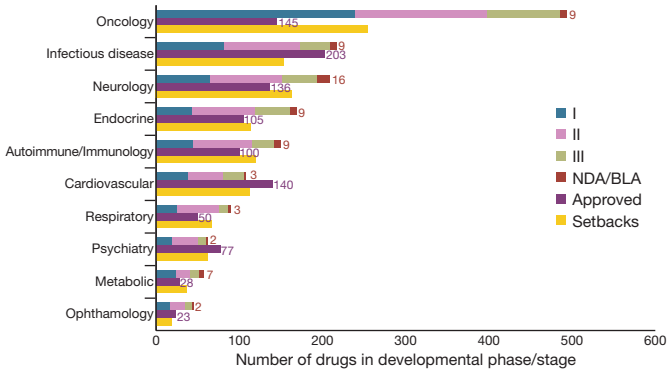
Drug pipeline: Q410

Wayne Peng

The first recombinant human protein made in transgenic rabbit milk was approved in October by the European Medicines Agency. Proof-of-efficacy data in human phase 2 studies were obtained for several nucleic acid therapies: OncoGenex Pharmaceuticals' custirsen and Hemispherx Biopharma's Ampligen (rintatolimod) met their endpoints, whereas Antisense Pharma's trabedersen showed only a clinical benefit. Elsewhere, results were positive for Vertex Pharmaceuticals' VX-770 in a phase 2 trial of cystic fibrosis patients.

Top ten disease grouped by pipeline size

Oncology drugs dominate the development pipeline.



Source: BioMedTracker, a service of Sagient Research

Notable trial results (September–December 2010)

Drug name	Company	Indication	Result summary
Teleprevir	Vertex Pharmaceuticals	Hepatitis C virus (HCV) infection	Final phase 2a trial results showed greater anti-HCV activity in combination therapy with peg-interferon and ribavirin (Amer. Assoc. Study Liver Dis., Abstract 828, 2010).
Boceprevir	Merck/Schering-Plough	HCV infection	Final phase 3 trial results met primary endpoint in combination therapy with peg-interferon and ribavirin (Amer. Assoc. Study Liver Dis., Abstract 216, 2010).
Iniparib	Sanofi-aventis/ BiPar Sciences	Breast cancer	Phase 2b study met primary endpoint, safety and tolerability, and showed significant clinical benefit for the poly ADP-ribose polymerase inhibitor (*N. Engl. J. Med.* doi: 10.1056/NEJMoa1011418, 2011).
Briakinumab	Abbott/Cambridge Antibody Technologies	Psoriasis	Phase 3 trial results for the fully human anti-IL-12/IL-23 mAb met two co-primary endpoints compared to placebo and Enbrel (European Academy of Dermatology and Venereology, 10/11/2010).
Custirsen	OncoGenex Pharmaceuticals	Prostate cancer	Phase 2 trial data of the clusterin-inhibiting antisense oligonucleotide met primary endpoint and showed significant survival benefit with docetaxel combined therapy (*J. Clin. Oncol.* **28,** 4247–4254, 2010).
Trabedersen	Antisense Pharma	Brain cancer	Phase 2b trial for the phosphorothioate transforming growth factor beta 2-specific antisense oligodeoxynucleotide failed to meet primary endpoint but showed clinically relevant benefit (*Neuro. Oncol.* doi:10.1093/neuonc/noq142, 2010).
VX-770	Vertex Pharmaceuticals	Cystic fibrosis	Phase 2 trial demonstrated safety of the small-molecule potentiator of the cystic fibrosis transmembrane conductance regulator and reached statistical significance for a secondary endpoint, sweat chloride concentration (*N. Engl. J. Med.* **363**, 1991–2003, 2010).
Ampligen (rintatolimod)	Hemispherx Biopharma	Chronic fatigue syndrome	Phase 3 trial of the experimental double-stranded RNA drug reduced the chance of long QT syndrome and alleviated medication dependence compared with placebo (*J. Applied Res.* **10**, 80–87, 2010).

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

Notable regulatory approvals (September–December 2010)

Drug name	Indication	Approval	Drug information
Gilenya (fingolimod)	Multiple sclerosis	9/21/10 FDA	Novartis' sphingosine 1-phosphate receptor agonist
Ruconest (EU)/ Rhucin (US) (conestat alfa)	Hereditary angioedema	10/28/10 EMA; PDUFA date 10/28/11	Pharming's recombinant human C1 elastase inhibitor produced from transgenic rabbit milk
Egrifta (tesamorelin)	HIV lipodystrophy	11/15/10 FDA	Theratechonologies' peptide fragment of human growth hormone releasing factor

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

Notable regulatory setbacks (September–December 2010)

Drug name	Company	Indication	Setback summary
Bydureon (exenatide LAR)	Amylin Pharmaceuticals	Type 2 diabetes	10/19/10 FDA issued a complete response letter requesting additional studies, delaying potential approval until 2011.
Numax (motavizumab)	MedImmune-AstraZeneca	Respiratory syncytial virus	12/21/10 AstraZeneca withdrew BLA for the humanized monoclonal antibody.
Zalbin (albinterferon alfa-2b; also known as Albuferon or Joulferon)	Human Genome Sciences/Novartis	Hepatitis C	10/4/2010 FDA issued a complete response letter rejecting the BLA. European MMA was withdrawn earlier on 4/19/10.
Simplirix	GlaxoSmithKline	Herpes simplex virus (HSV) infection	9/30/10 Development for vaccine comprising HSV glycoprotein subunit gD2 and adjuvant SBAS4/AS04 stopped after missing primary endpoint of phase 3 trial.
Vismodegib	Genentech/Roche	Ovarian cancer	10/11/10 Development of Smoothened antagonist stopped after missing primary endpoint in phase 2 trial.
ISIS 369645	Isis	Asthma	11/4/10 Company discontinued development of antisense drug due to insufficient improvement in phase 2a trial.
TroVax	Oxford Biomedica	Renal cell cancer	10/14/10 Phase 3 trial for vaccine comprising tumor antigen 5T4 failed to meet overall survival primary endpoint (*Clin. Cancer Res.* doi:10.1158/1078-0432, 2010).

Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com/). BLA, Biologic License Application; LAR, long-acting release; MMA, marketing authorization application.

Notable upcoming decisions (Q1 2011)

Drug Name	Company	Indication	Expected approval decision
Sopura/Trizytek (liprotamase)	Eli Lilly	Exocrine pancreatic insufficiency	1/31/10 PDUFA. Oral recombinant enzyme replacement therapy that contains lipase, protease and amylase.
Esbriet (pirfenidone)	InterMune	Idiopathic pulmonary fibrosis	2/1/11–3/31/11 MAA decision date range. Phase 3 trial results of the p38 kinase inhibitor demonstrated dosage-dependent response and safety. European Respiratory Society, 9/19/10, Abstract 388.
Uplyso (taliglucerase alfa)	Protalix BioTherapeutics/ Pfizer	Gaucher disease	2/25/11 PDUFA date. Recombinant human glucocerebrosidase demonstrated both safety and effectiveness, according to company press release on 11/2/10.

Other expected approvals in Q1 delayed from the previous quarter include: Human Genome Sciences' Benylsta (belimumab), Madarex/Bristol-Meyers Squibb's Yervoy (ipilimumab), MannKind's Afrezza (inhaled insulin) and LG Life Sciences' LB03002 (RS-rHGH).
See *Nat. Biotechnol.* **28**, 998, 2010 for details.
Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com/).
PDUFA, Prescription Drug User Fee Act. MMA, market authorization application.

Wayne Peng is Emerging Technology Analyst, Nature Publishing Group