NEWS IN BRIEF

Biotech R&D spend jumps by more than 15%

Two reports show big increases in R&D spending among both biotech and pharmaceutical drug developers.

In an annual review of the biotech sector, analysts at EY (formerly Ernst & Young) found that biotech companies spent US$40.1 billion on R&D in 2015, up 16% from their 2014 spend (see FIG. 1). For the second year in a row this increase was led by the sector's smaller companies, which cumulatively increased their R&D budgets by 28% (to $15 billion). Established companies with revenues of at least $500 million per year increased their R&D spend by a lower level of 10%. R&D expenses grew more quickly than revenues, the analysts write, "suggesting a continued willingness to bet on the industry pipeline."

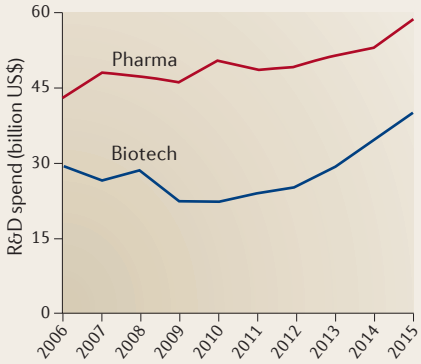

Figure 1 | **R&D spend.** Biotech R&D spending data are from the EY Biotechnology Reports 2008–2016. Where annual reports provided inconsistent R&D spending data, data from the latest report were used. Pharmaceutical R&D spending data are from the PhRMA 2016 Profile.

The analysts note that, although the biotech sector enjoyed a record performance in 2015, revenue and market cap growth slowed in 2015. These data suggest that "biotech's wave of unprecedented success may have crested," they write.

A separate report by the industry lobby group PhRMA, meanwhile, showed that pharmaceutical companies spent $58.8 billion on R&D in 2015, up 10% from their 2014 spend.

The R&D budgets of some companies are captured in both cohorts.

Asher Mullard

Parsing clinical success rates

Conventional wisdom holds that only around 10% of drug development projects make it all the way from Phase I to approval. Two studies of clinical trial success rates now provide updated granularity to this rule of thumb, and show considerable variation by therapeutic area and drug modality.

In a first report, the industry lobby group BIO, along with analysts at BioMedTracker and Amplion, analysed 7,455 drug development programmes that moved through the clinic between 2006 and 2015. They found that the probability of success was 63% in Phase I trials, 31% in Phase II trials, 58% in Phase III trials and 85% during the regulatory review process, for an overall success rate of 9.6% (63% × 31% × 58% × 85% = 9.6%). But when they analysed the data by therapeutic area, the overall success rates ranged from 26% for haematology projects to 5% for oncology projects (see FIG. 1).

Rare disease drugs for non-cancer indications out-performed the average, with an overall success rate of 25%. Projects that included biomarkers fared similarly well, achieving an overall success rate of 26%.

The overall analysis lumped new molecular entities (NMEs; including small molecules and antibodies) together with 'non-NME' development projects such as reformulations and combinations of approved drugs (but not generics). When the analysts focused on the NMEs to assess only the most innovative therapies, the overall success rate was 6.2%. Novel biologics, including antibodies and gene therapies, performed better, with an overall success rate of 11.5%.

A second study shed further light on the clinical trial success rates and provides cause for optimism. Analysts at the consulting firm McKinsey & Company tracked the progress of 9,200 compounds that were developed between 1996 and 2014. When they calculated a rolling 3-year average, they found that success rates are on the rise. They calculated a cumulative success rate of 11.6% in 2011–2014, up from a low of 7.5% in 2008–2011 (*Nat. Rev. Drug Discov.* **15**, 379–380; 2016).

Several of their findings mirrored those of the BIO study. McKinsey calculated an above-average overall success rate for rare disease drugs, hitting 29% over the past 3 years. Biologics had an overall success rate of 18%, twice that of the 9% success rate for small-molecule drugs.

Asher Mullard

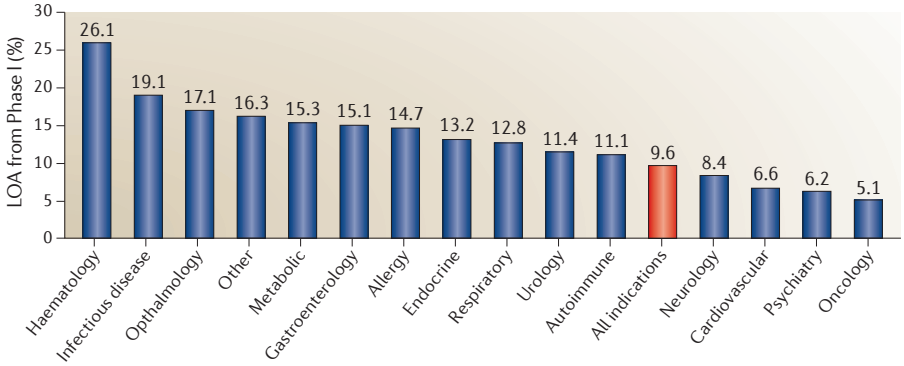

Figure 1 | **Likelihood of approval (LOA) from Phase I.** Data from Clinical Development Success Rates 2006–2015 by BIO, BioMedTracker and Amplion.

EMA provides first glimpse of PRIME candidates

In March, the European Medicines Agency (EMA) launched the PRIME programme, a variation of the FDA's breakthrough designation programme aimed at speeding up the development of promising medicines with high potential to address unmet needs. They have now disclosed the first four PRIME candidates. Only one of these drugs (KTE-C19) has been publicly disclosed as having breakthrough designation.

The first four PRIME candidates are: Biogen's aducanumab, a beta-amyloid targeting antibody for Alzheimer disease; Kite Pharma's KTE-C19, a chimeric antigen receptor (CAR) T cell therapy for diffuse large B cell lymphoma; ChemoCentryx's CCX168, a C5a receptor inhibitor for a set of rare autoimmune diseases called ANCA-associated vasculitis; and Novimmune's NI-0501, an anti-interferon-γ antibody for the rare autoimmune disease haemophagocytic lymphohistiocytosis.

The agency denied PRIME designation to 14 submissions.

The EMA will disclose further PRIME candidates on a monthly basis.

Asher Mullard