Table 1 Selected read-through drugs in development

Company	Lead drug	Mechanism	Indications	Clinical status
PTC Therapeutics	Ataluren	R≠ead-through of premature termination codons	DMD Cystic fibrosis	Filed (Europe)[a] Phase 3
Prosensa, GlaxoSmithKline (London)	Drisapersen	Antisense oligonucleotide promotes exon 51 skipping	DMD	Phase 3
Sarepta Therapeutics	Eteplirsen	Antisense oligonucleotide promotes exon 51 skipping	DMD	Phase 2b

[a]PTC is seeking conditional approval prior to readout of a phase 3 trial in DMD.

between signals arising from the target biology versus the underlying assay technology itself." The two detection systems employed are mechanistically unrelated, so an artifact arising out of two different events is highly unlikely (*Nat. Methods* **9**, 937, 2012).

The publication of McElroy's paper did some short-term damage to the PTC share price—the stock fell by 22% during the two days after its publication—but it recovered quickly and attained a peak of $18.50 on 16 July, having been priced at $15 per share in the initial public offering (IPO). However, the market has responded with greater enthusiasm to Prosensa, a newly listed Dutch firm that is targeting a different population of DMD patients—those with Stargardt's disease—with an exon-skipping antisense drug drisapersen (**Table 1**). One week after PTC's debut, Leiden-based Prosensa priced its IPO on NASDAQ at $13 per share. The stock closed on August 6 at $30.01, valuing the company at over $1 billion, whereas PTC was valued at $362 million on the same date.

Prosensa's oligomers target RNA sequences involved in RNA folding or in recognizing serine-arginine (SR) proteins, which play numerous roles in mRNA splicing and translation. The company uses DNA sequence information to identify which exons—the dystrophin protein contains 79 in all—are potential candidates for read-through, in order to correct frameshifts caused by genetic deletions of one or more exons. "It's the exons flanking the mutation that you skip," says Prosensa's vice president of drug discovery, Judith van Deutekom. "The reading frame is correct, and you can make a protein again." Although the resulting dystrophin protein is truncated, it appears to lead to a milder phenotype, which is similar to a form of the condition called Becker's muscular dystrophy. Drisapersen is suitable for around 13% of DMD patients. The company has six other development programs at earlier stages of development, which will broaden its coverage to more than 40% of the patient population.

Cambridge, Massachusetts–based Sarepta Therapeutics (formerly AVI Biopharma), which is following a similar strategy, also has a market value in excess of $1 billion. Sarepta recently announced its intent to file an NDA for its lead DMD drug eteplirsen during the first half of 2014, based on data from a twelve-patient phase 2 study. After 84 weeks, patients treated with the drug gained a 46.4 meter advantage in a six-minute walk test (6MWT) over those treated with placebo for an initial 25 weeks, who were then rolled over into an open-label extension study in which all participants received the drug. At 48 weeks, the difference between the two groups was 67.3 meters. Before the end of this year, Prosensa expects to report data from a phase 3 study of drisapersen in 180 patients. It previously reported data from a 53-patient exploratory phase 2 trial, in which those on the drug gained a 35.1 meter advantage in the 6MWT after 24 weeks of treatment over those on placebo. The gap was maintained after 48 weeks of treatment, when the average difference between the two groups was 35.8 meters. The future utility of all of these drugs will, of course, be determined by their safety and efficacy profiles rather than the fine details of their biological activities. The arrival of the data that will determine their clinical potential is imminent.

Cormac Sheridan *Dublin*

Correction

In the version of the article entitled "Galecto Biotech" (*Nat. Biotechnol.* **31**, 481, 2013) originally published, in paragraph 2, "Galectin-3" was said to be "like," but is "unlike other lectins." The photo caption should have included Hans Schambye, also a co-founder; and Tariq Sethi's first name was misspelled as Tarik. At the end of the article, Raghu Kalluri's name was misspelled as Khalluri. The errors have been corrected in the HTML and PDF versions of the article.

IN brief

Synthetic biology goes industrial

Imperial College London has won a £10 ($16)-million grant to set up a new translational center aimed at integrating academic and industry research in synthetic biology. Known as SynbiCITE, the London-based center is funded by the Engineering and Physical Sciences Research Council, Biotechnology and Biological Sciences Research Council and Technology Strategy Board. So far, the new center brings together 17 other UK universities with 13 corporate partners, including heavy hitters such as Microsoft, GlaxoSmithKline, Syngenta, Shell and Agilent Technologies, as well as small-to-medium enterprises. According to SynbiCITE's co-director, Paul Freemont, the research will be focused and milestone-driven in a close collaboration with industry partners to reflect their needs. He predicts that industry will be most interested in new products and tools that speed up pathway engineering, bringing the latest genomic and metagenomic information to bear on the problem of producing useful materials quickly and to scale. Stephen Laderman, director of the Molecular Tools Laboratory at Agilent Technologies, says that the new venture is exciting and that industry is keen to engage, with the expectation that engineered organisms, components and design methods with innovative applications will result. Companies, he notes, are already benefitting from synthetic biology advances, especially in the area of bioprocessing. Freemont says that the venture is open to other partners who did not come in on the first round. All partners, industrial as well as academic, commit to fund the venture both in cash and in kind. Although it is impossible to predict the most successful outputs, Freemont says that by working closely together, academic and industrial researchers can ensure commercial needs are aligned with the research. Last year, synthetic biology was included as a priority in the UK government's science package for 2015 (*Nat. Biotechnol.* **31**, 93, 2013). *Jennifer Rohn*

IN their words

"It's incredibly ironic that this is a transparency initiative and we've now got clear indications that the pharmaceutical industry is ready to use patient organizations to fight [in] their corner." Tim Reed, of Health Action International, commenting on pharmaceutical companies, efforts to resist the release of clinical trial data being argued in the EU. (*The Guardian*, 21 July 2013)