

Roche buy-in is another vote of confidence in gene therapy



Jacob Plieth



The Swiss group picks up Spark Therapeutics for \$4.8bn, marking the biggest gene therapy buyout since Novartis's \$8.7bn move on Avexis.

Concerns about pricing and treatment durability have undermined the economic viability of gene therapies, but they have not stopped Pfizer, Novartis, Johnson & Johnson and Abbvie from buying in. Today Roche gave the field another huge endorsement, taking over Spark for \$4.8bn in a bid to consolidate its haemophilia A franchise.

The move shows that the Swiss group remains an active acquirer of small biotech companies, notwithstanding its protestations about being a disciplined buyer. In the near term it sets up a mid-year showdown between SPK-8011 and a rival from Biomarin, whose stock traded up 4% this morning.

This was the result of improving sentiment about gene therapy in general as, in reality, Roche backing a rival might not bode well for Biomarin. Other gene therapy-focused biotechs on the rise this morning included Unique (up 41%), Audentes (15%) and Regenxbio (10%). The Spark takeover comes a year after Roche bought Ignyta at a 74% premium for \$1.7bn.

Gene therapy followers in particular will note that [just last Friday](#) Abbvie paid Voyager \$65m up front to expand a gene therapy tie-up. Roche's Spark move is the largest gene therapy takeout since Novartis paid \$8.7bn for Avexis.

Secret sauce

So what was it about Spark and Avexis that turned these companies into must-have assets? Evercore ISI analysts wrote today that both companies had three things in common: a validated asset, proven manufacturing expertise and a pipeline.

Spark has the first ever US-approved gene therapy, Luxturna; but this targets a niche condition – biallelic RPE65 mutation-associated retinal dystrophy – and is already partnered with Novartis outside the US. Sellside consensus gives it an NPV of just \$726m, according to *EvaluatePharma*.

The focus for Roche must therefore be haemophilia A and SPK-8011, which delivers the gene for factor VIII using an AAV vector. Interestingly, this asset has disappointed at the last two Ash conferences, [revealing continued cases of immunogenicity](#) that Spark will attempt to combat by using prophylactic steroids.

This puts the spotlight on the mid-year announcement of data from a phase I/II expansion cohort, showing whether prophylactic steroids can improve SPK-8011's safety and efficacy profile. Crucially, at around the same time Biomarin will reveal three-year data with valoctocogene roxaparvovec (valrox), a rival gene therapy that has so far outclassed SPK-8011 largely thanks to its possibility of being dosed at a much higher level.

However, there have been questions about valrox's durability ([Snippet roundup: Biomarin gets a double dose of doubt, 25 May 2018](#)).

Analysts expect Roche to have had a sneak peek at the latest SPK-8011 data, something that if true might not bode well for Biomarin. The broader question might be how the Spark assets fit in with Roche's existing haemophilia A offering, Hemlibra, a bispecific antibody whose sales could this year approach \$1bn.

Hemlibra is approved in patients with inhibitory antibodies and as prophylaxis therapy in those without inhibitors. In addition to various early-stage rare disease assets Spark's pipeline includes SPK-8016, a separate gene therapy for haemophilia A patients with inhibitors.

Other bids?

Investors following the recent uptick in biotech M&A will note that, unlike Tesaro and Immune Design, Spark is not being taken out after falling from grace and seeing its stock become affordable. Roche's \$114.5 bid represents a 126% premium to Friday's closing price, and is 24% above Spark's all-time high, hit last year.

The premium Roche is paying for Spark suggests that bidding was competitive, but Spark's closest partner, Pfizer, paradoxically seems unlikely to have expressed an interest.

Pfizer is the licensor of Spark's haemophilia B gene therapy, SPK-9001, but in haemophilia A it is tied to a separate factor VIII asset, Sangamo's PF-07055480. Despite the clear threat that the Roche bet now poses, Sangamo climbed 5% today as the rising tide lifted most gene therapy boats.

The market seems to have decided that, as Evercore suggests, a \$4.8bn valuation for Spark makes "virtually everything else in gene therapy look exceedingly cheap". The less sophisticated view is that Roche has felt a sudden need to catch up with its Swiss rival Novartis.