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Who loves ya, Baebies? The FDA does

Jonathan Gardner

First came treatments for lysosomal storage disorders, and now come the infant screenings. Baebies has received the first ever US FDA approval for a test to detect four such metabolic diseases from dried blood spots taken from newborns.

With lysosomal storage disorder testing now required in 11 US states, Baebies could have a ready-made market waiting for it on commercial launch. The four rare conditions are the speciality of Sanofi's Genzyme division, providing a reason why the French group might want to acquire Baebies to bundle screening and treatment together for value-minded purchasers.

They call me the Seeker

The Seeker system tests for the conditions mucopolysaccharidosis type I (MPS I), Pompe, Gaucher and Fabry diseases – genetic conditions that if untreated can cause organ damage and death owing to an accumulation of unmetabolised molecules. Since the early 1990s, numerous products have been launched to treat them, turning many into multi-billion-dollar markets.

Baebies sought approval based on a review of blood spot specimens collected by the Missouri State Public Health Laboratory. Seeker had a false positive rate of less than 1% in all four conditions, while no false negatives could be identified through follow-up with metabolic centres.

The Seeker diagnostic is Baebies's first bid to enter neonatal testing, with the follow-up Finder hoped to reach commercial sale sometime in 2017. The North Carolina-based group has suggested that this point-of-care technology could initially be used for hyperbilirubinemia.

These diagnostics rely on digital microfluidics technology licensed from Illumina, which acquired it from Advanced Liquid Logics. Baebies hopes that the technology will be able to perform screening tests with smaller blood samples than currently used.

Part of the franchise

North Carolina-based Baebies might have found itself in a therapeutic sweet spot. Sales of lysosomal storage disorder agents have grown rapidly, nearly tenfold since 2000, and are forecast to grow 8% a year from 2016 through 2022.

As orphan conditions, they have been relatively free of pricing pressure – Sanofi's Fabrazyme, on track to become a blockbuster treatment in 2022, cost $361,000 a year in 2016. Yet with fast growth in these categories payers are likely to become more sensitive in coming years.

Besides Sanofi, Shire and Biomarin have been active in treatments for lysosomal storage disorders. When entering pricing negotiations with payers, having diagnostics and treatments would offer a competitive advantage, especially in the harder-fought categories like Fabry and Gaucher diseases.

Furthermore, early detection could present an opportunity for value-based pricing as it could help avert downstream healthcare costs. Sanofi has already signed a value-based deal in the US on its cholesterol lowering drug Praluent.

Of course, buying a diagnostic might be a big leap for any of these companies, and would represent a reversal for Sanofi, which sold off its diagnostics joint venture with Institut Pasteur in 1999. But since Baebies's one disclosed fund-raising was a $13m round in 2013, this could be a cheap way of protecting a rare disease franchise.

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