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Rare But Real: How One Startup Is Willing Itself To Lift China Orphan Drug Market

21 Jan 2019 | **ANALYSIS**

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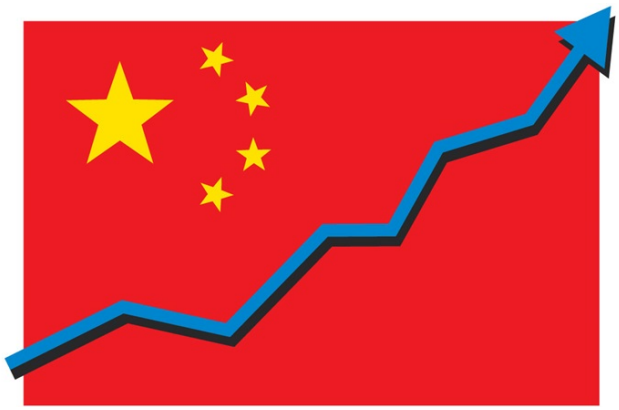
Executive Summary

As a promising market for orphan drugs, China has seen several approvals in this space in the past six months, mostly from multinationals. But Beijing-based CANbridge believes that a smaller biotech also has a good shot at a leadership role, despite a lack of funding and reimbursement and prickly pricing issues.



DESPITE CHALLENGES, CANBRIDGE'S XUE SEES OPPORTUNITIES FOR CHINA ORPHAN FIRMS

Source: Shutterstock

CANbridge Life Sciences Ltd. has been front and center about its desire to accelerate the development of novel and orphan drugs in China.

To this end, in the first few days of the new year, the Beijing-based startup had already bagged two new deals in the orphan space, one being an expansion of its collaboration with WuXi Biologics for four potential assets for rare genetic conditions. (Also see "Deal Watch: Denali Partners With Sirion To Take On The Blood-Brain Barrier" - Scrip, 11 Jan, 2019.)

The other was a licensing agreement for exclusive rights to South Korean company GC Pharma's (formerly Green Cross Corp.) Hunter syndrome therapy, *Hunterase* (idursulfase-beta), which is already marketed in over 10

countries. (Also see "Asia Deal Watch: Boost For Esperion's Cholesterol Candidate As Daiichi Brought On Board" - Scrip, 8 Jan, 2019.)

CANbridge's strong drive for orphan drug development in China can be put down largely to founder, chairman and CEO James Xue's professional background. After a stint as an executive at global biotech Genzyme Corp., he started CANbridge with a mission to introduce novel oncology products to China.

Scrip caught up with Xue at an early December meeting on rare diseases held in Shanghai, during which he discussed topics ranging from orphan development challenges in China to likely pathways to move forward, both in a presentation and on the sidelines.

China Orphan Challenges

"There are many drugs there have been approved in the US but not in China," Xue noted in his presentation to the meeting, pointing to two major remaining challenges for orphan drug developers in the country: one being the lack of commercial market and funding, and the second a shortage of policy incentives such as tax breaks and market exclusivity.

China's policies for orphan drugs not only lag behind developed countries such as the US, but are under-developed even compared to similar emerging markets such as Brazil. In this largest South American country, access to 119 orphan drugs is already covered via negotiations between the government and manufacturers, but so far only limited coverage is available in China for certain treatments.

At the latest tally, only six Chinese provinces and cities had any form of coverage for certain rare pediatric conditions.

However, this doesn't mean that smaller firms don't have any opportunities, and Xue noted that, out of 26 orphan drugs approved by the US FDA from 2017-18, fully one third were developed by companies that were only created in the recent 15 years. "Many startups of today are potentially stars of tomorrow," Xue said.

The executive believes collaboration with like-minded companies is one sure way to accelerate orphan drug development in China. To this end, CANbridge formed and expanded its tie-up with WuXi Biologics, aiming for the first IND filing under this in China in 2019, while Xue, along with the Beijing Union Hospital and China Pharmaceutical R&D Association (SinoPhiRDA) formed the China Rare Disease Alliance in December.

Bring Them On: 40 Orphan, Cancer Drugs Set For Priority Review In China

By Brian Yang

06 Nov 2018

New treatments for spinal muscular atrophy, glioblastoma, Gaucher's disease and others could be soon be hitting the China market following the awarding of priority reviews to a large list of novel therapies.

Read the full article here ›

In the meantime, the company has several licensed-in assets in late-stage development. The most advanced is *Nerlynx* (neratinib; licensed from Puma Biotechnology Inc.) for early-stage breast cancer, which is pending the approval in China. Another is the oncology drug CAN008 (licensed from Apogenix GMBH), an antibody-like CD95 receptor/Fc-fusion protein for glioblastoma in Phase II/III development.

Industry Needs To Step Up

While the Chinese government needs to step up to provide new policy incentives in the area, in particular for reimbursement coverage and the timely inclusion of new drugs into China's National Reimbursement Drug List (NRDL), the industry also needs to persuade local governments to start pilot schemes for such products, Xue suggested to *Scrip* at the Shanghai meeting.

Currently, the national reimbursement list is updated only every one to two years. The best approach would be for a combination of a dynamic adjustment of the NRDL, local pilots for certain appropriate regions, and efforts from both the industry as well as individual patients to get orphan drugs approved, Xue suggested.

The pricing of such products is another thorny issue. The government in China has implemented a massive and ambitious centralized drug bidding scheme in major cities, the so-called "4+7" scheme, which has cut the prices of some widely prescribed drugs by 52% on average, and by up to 96%.

Xue said that in the case of pricing for orphan drugs, this must be "reasonable", making them more widely accessible but also allowing for profits by suppliers. Reflecting his experience at Genzyme, the executive noted that one product can also give birth to an entire ecosystem in the orphan sector, such as in the case of *Cerezyme* (imiglucerase for injection).

Since the first launch of the treatment for type 1 Gaucher Disease, a cluster of biotechs was spurred into action around Genzyme's Boston base, resulting in a blossoming of investment, technology and products.

Could a similar process started by a smaller biotech company take place in China? Xue seems to believe so.

From the editors of PharmAsia News.