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Interview – Santhera readies muscular dystrophy push

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In Duchenne muscular dystrophy one name has received nearly all the attention: Sarepta Therapeutics, which persuaded the US FDA to approve its Exondys 51 over objections that its supporting data were inadequate. Switzerland's Santhera Pharmaceuticals believes that its entry, Raxone, ought to be receiving at least as much attention.

Raxone's Delos clinical trial is "still the only successful phase III trial in Duchene muscular dystrophy", its chief executive, Thomas Meier, notes. So far, it has not resulted in a broad launch, but this could change very soon with an EU advisory committee decision due later this year.

This is a bold statement, to be sure, but Mr Meier follows it up with ambitious commercial plans. "We have taken a position that we are not interested in licensing. We would like to keep the rights," he says, pointing to the example of its fellow Swiss group Actelion as a "prominent success case next door" for going it alone in speciality medicine.

Not the same

Beyond that they are both hoping to enter Duchenne muscular dystrophy (DMD) in quick succession, there are few comparisons between Raxone and Exondys, and they will not compete. The latter seeks to delay the disability requiring the use of wheelchairs in young DMD patients with a specific mutation. The Swiss group's agent, meanwhile, aims to support respiratory function in older patients already in wheelchairs.

The Delos trial was able to show that patients taking Raxone experienced a non-significant 3.05% decline versus baseline in peak expiratory flow in 52 weeks of treatment, compared with the significant 9.01% decline experienced by patients taking a placebo.

This trial took place in patients not using corticosteroids to maintain respiratory function. Before approval, US FDA officials wanted to see data in patients who use corticosteroids. That trial, called Sideros, will enrol 266 subjects – more than four times the number in Delos – and will measure respiratory function after 78 weeks of treatment. Mr Meier says it will read out in late 2019.

The decision first to test the pill in the non-steroid population was based on disease progression. "Sometimes patients become intolerant. This is where the medical need was highest," he says.

Delos was sufficient for the European Medicines Agency to accept an application, and a recommendation from its human medicines committee is due in weeks. In the meantime, the UK's MHRA has allowed Raxone into its Early Access to Medicines Scheme, giving DMD patients not on corticosteroids a new treatment option.

In addition, the drug has already been approved in the EU for the orphan indication Leber's hereditary optic neuropathy (LHON), giving it a commercial infrastructure on which to build. Santhera reported sales of SFr19m ($19.6m) in 2016, with 280 patients using the agent at year end.

The existence of supplements sharing the same name as Raxone's active ingredient, ibedenone, does not faze Mr Meier. Having the regulatory approval in Europe already, along with orphan designations in both the EU and US, should offer it exclusivity – and having commercial sales in LHON would support his belief that Raxone will be protected.

Having approvals in two different indications with different clinical outcomes – in LHON, improvement in visual acuity and in DMD respiratory function – raises some issues with payers. For example, Germany's IQWIG has set indication-specific prices, but will reimburse for Raxone on a volume-weighted average of the two. The issue will become even more complex should work in primary progressive multiple sclerosis bear fruit and yield regulatory approvals.

Ready for launch

Santhera sold SFr60m ($62m) worth of convertible bonds earlier this year, giving it a cash position of SFr100m,

which Mr Meier says will back the DMD launch. In preparation for a move to the US, the group has six people in position and plans to have “12 or so” by the end of the year – it opened an office in Massachusetts this year.

Following Raxone, Santhera is conducting a phase I trial of omagapil in paediatric and adolescent patients with congenital muscular dystrophy. This is a compound licensed from Novartis, which tested it in Parkinson’s disease and amyotrophic lateral sclerosis. In Santhera’s hands, it has been converted from a pill to a liquid formulation that can be used more conveniently in babies and can be dosed by weight.

A pharmacokinetic clinical trial is being run that could have results later this year.

Should Santhera launch Raxone launch successfully, it could be looking at developing new agents to keep the pipeline flowing, much as its neighbour Actelion did before being acquired by Johnson & Johnson. But Mr Meier says the group will remain focused on neuromuscular and mitochondrial diseases like muscular dystrophy or Friedreich’s ataxia.

“We’re not a cancer company. But there are plenty of interesting opportunities,” Mr Meier says.

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