

Mesoblast sows the seeds for stem cell success

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Chief executive Silviu Itescu says Mesoblast is on track to launch the world's first stem cell product next year *Source: News Corp Australia*

MESOBLAST chief executive Silviu Itescu says the pioneering regenerative medicine company is on track to launch the world's first stem cell therapy product in Japan next year and this could be followed by a US launch in 2016.

Mr Itescu said the likely launch in Japan — which is becoming a centre of importance for cell therapies thanks to regulatory changes — would see the company massively re-rated and trigger a revaluation of the fledgling cell therapy industry.

So far no company or organisation has won approval for a stem cell therapy product in a developed world market, but through its deal with Japan's JCR Pharmaceuticals, Mesoblast is hoping to be the first to crack that nut.

The ASX-listed company — backed by prominent investor Alex Waislitz and other high-profile individual and corporate investors — is currently worth \$1.3 billion, but is costing \$80 million a year to run as it pushes towards a market launch.

Mesoblast has been a bumpy ride for many investors with the stock price hitting a high of \$9.14 in October 2011 before retreating gradually to a low of \$4.10, where it closed on Friday.

The price fell 27c — or 6 per cent — yesterday after Goldman Sachs issued a rare sell rating on the stock, saying development risks had not been fully factored into the price.

But in an interview at the company's Collins Street HQ in Melbourne last week, Dr Itescu told *The Australian* he was not preoccupied with the share price or market capitalisation.

With the company set to have three products in phase III trials (the final phase before approval) next year and hoping to have two products in the market in 2016, he believes the price will take care of itself. "I don't like to comment on share prices, market capitalisations and things like that. There continues to be volatility in our stock price. I am focused first on getting products into the marketplace," he said.

"I think we are pre-revenue and there remains risk, and I think there remains substantial upside. If any one of our products demonstrates success during phase III, you will see this company massively rerated given the market opportunities for any one of our lead products and their future revenue streams.

"Until the first product is approved, nobody can be certain that this is going to be a real industry. When that happens, you will have a major crystallising event that will have a flow through on valuations of all companies in the cell therapy space, not just us."

Mesoblast has put itself on the verge of being the first mover in the cell therapy game thanks to a deal with JCR, the Japanese company that is seeking approval to use Mesoblast's mesenchymal stem cell technology to treat graft-versus-host-disease (GVHD), a potentially fatal complication of bone marrow transplants.

Japan, with its ageing population and technological prowess, is eyeing cell therapy as a major industry and has launched a fast-track approvals process for cell-based treatments under reformist Prime Minister Shinzo Abe.

"That means, in effect, the ability to launch our products in Japan is shortened in terms of time, and reduced in terms of cost," Dr Itescu said.

"It would probably shave off about three years per product. To be able to launch a product off relatively smaller phase III trials is very advantageous for a company like Mesoblast."

The breakthrough that investors have long hoped for is expected to come in 2015 when Japanese regulators are expected license the GVHD product for sale.

"To date, no company has developed an adult off-the-shelf manufactured stem cell product in a first world country — completed, launched, marketed. I think to be the first to do that, be it in Japan or in the US, would be a tremendous step forward for any company, let alone a public company out of Australia," Dr Itescu said.

Although Japan is an important market, Dr Itescu said the US was always the key target market for the company. Phase III trials have been completed using Mesoblast's mesenchymal stem cells for treating childhood GVHD in the US and the company is in talks with the US Food and Drug Administration about fast-tracking approval, with the aim to go on sale in 2016.

It also plans to commence a trial and seek approval to treat adult GVHD. The company is in advanced trials of this product for treatment of Crohn's disease.

While Mesoblast hopes the mesenchymal stem cells used for the GVHD treatment will bring the first cash through the door, Dr Itescu has said consistently that the portfolio of treatments using mesenchymal precursor cells will probably prove more valuable in the long run.

The company has a product aimed at repairing cardiac muscle damage from congestive heart failure in a phase III trial in the US and intends to commence a phase III trial of its degenerative disc repair product by the end of this year.

Mesoblast, with some \$220m in the bank, has sufficient cash to fund the degenerative disc repair trial, while its Israel-based partner Teva Pharmaceutical Industries is funding the heart failure trial in exchange for a split of future revenues.

"Both of those (products) address significant unmet needs," Dr Itescu said.

"The heart failure product is targeted at 40 per cent of the heart failure population — those with muscle loss."

The disk failure product is aimed at patients for whom spinal fusion surgery is the only other alternative.

"Having three products in late phase (trials) reduces the risk. That gives us multiple shots on goal for success," Dr Itescu said. "They are not all going to work necessarily, but you only need one to make it through ... and you have a successful biopharma company."

Biotech hopefuls typically have to scale up manufacturing even before approval is granted. Mesoblast has established plants in the US and Singapore and will decide in the next nine to 12 months whether to have a factory in Australia, and in which state that might be.

"Obviously it would make most sense to be Victoria — it's where our corporate headquarters is — everything being equal," Dr Itescu said.

Goldman Sachs, in the research note that preceded yesterday's price slump, reduced its expected price for Mesoblast from \$5.50 to \$3.20, citing development risks that were not priced into the stock.

In response, the company said it believed Mesoblast had never been in better shape.

At the other end of the spectrum, Bell Potter has a \$9.43 speculative buy rating on the stock.

Analyst Tanu Jain said the company was undervalued.

