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FDA Approves Mesoblast's Ryoncil For GvHD

Mesoblast says the US Food and Drug Administration has approved Ryoncil for children aged two months and older for graft versus host disease (GvHD).

Mesoblast said the Ryoncil (remestemcel-L, previously known as MSC-100-IV) approval included adolescents and teenagers, with steroid-refractory acute graft versus host disease (SRaGvHD), a life-threatening condition with high mortality rates.

In 2018, Mesoblast said 41 of 55 children (74.5%) in its open-label, phase III trial of remestemcel-L for acute graft versus host disease survived to 100 days, and later said that 38 of 55 children (69%) survived to 180 days (BD: Jun 21, Sep 30, 2018).

In 2020, the FDA said it required a further trial of remestemcel-L and "recommended that [it] conduct at least one additional study in adults and/or children (BD: Oct 2, 2020).

Last year, Mesoblast said the FDA provided a second complete response letter requiring more data; and later said it expected to contract a pivotal trial (BD: Aug 4, Nov 15, 2023).

This year, the company said the 2018 phase III study data "appears sufficient" for a biologics' application for Ryoncil for paediatric steroid-refractory acute graft versus host disease and it resubmitted its application to the FDA (BD: Mar 26, Jul 9, 2024).

Today, Mesoblast said "Ryoncil's immunomodulatory effects, including inhibition of T-cell activation and secretion of pro-inflammatory cytokines, position the therapy for potential other indications in diseases with excessive inflammation … [and Ryoncil was] the only MSC therapy approved in the US for any indication".

In 2013, Mesoblast said it would acquire the mesenchymal stem cells assets of the Maryland-based Osiris for up to $US100 million in cash and scrip (BD: Oct 11, 2013).

Today, Duke University Medical Center's Dr Joanne Kurtzberg said that SRaGvHD was "a devastating condition with an extremely poor prognosis".

"From today we are able to offer Ryoncil, the first FDA-approved treatment which will be life saving for so many children and will have a great impact on their families," Dr Kurtzberg said.

Mesoblast said that 10,000 US patients a year underwent allogeneic bone marrow transplant, of which 1,500 were children, and about 50 percent develop aGvHD and almost half of those do not respond to steroids, the recognized first-line treatment.

Mesoblast chief executive Prof Silviu Itescu said the company was "very pleased that the FDA has granted approval of Ryoncil and are proud of the company's commitment to the GvHD community in bringing this important new treatment to children and families with no other acceptable options".

"With Ryoncil approval by FDA, Mesoblast has demonstrated the ability to bring the first [mesenchymal stromal cell] product to market," Prof Itescu said.

"We will continue to work closely with FDA to obtain approval of our other late-stage products, including Revascor for cardiovascular diseases and rexlemestrocel-L for inflammatory pain indications, as well as expanding the indications for Ryoncil in both children and adults with inflammatory conditions," Prof Itescu said.

Mesoblast said that Ryoncil would be available in the US at transplant centres and other treating hospitals "in the coming weeks".

An attached media release from the FDA said that remestemcel-L had fast track, orphan drug and priority review designations.

The FDA said that the recommended dose was 2.0 x 10^6 MSC/kg body weight per intravenous infusion given twice a week for four weeks for a total of eight infusions, administered at least three days apart.

Treatment may be continued based on response at 28 days.

In late November, Mesoblast's market capitalization surpassed $2.0 billion.

Mesoblast was up $1.07 or 54.0 percent to $3.05 with 57.8 million shares traded.