Antisense jumped 100 percent on news that preliminary results from six patients dosed with ATL1102 for Duchenne muscular dystrophy indicate a “positive drug effect”.

In July, Antisense says five of the nine patients between 10 and 18 years of age had completed the 24-week dosing phase in its phase II trial of ATL1102 for Duchenne muscular dystrophy (DMD) at Melbourne’s Royal Children’s Hospital. (BD: Jul 24, 2019).

Today, the company said that six patients had completed dosing and preliminary data was “indicative of a positive drug effect of ATL1102 at the dose tested both at an immunomodulatory, that is effects on relevant immune cells, and disease progression, that is effects on muscle strength and function, levels.

Antisense said ATL1102 was an inhibitor of CD49d expression on certain immune cells such as T-cells and DMD patients with a greater number of T-cells with high levels of CD49d expression had more severe and rapid disease progression.

The company said the primary endpoints related to the safety and tolerability of ATL1102 and efficacy assessed in terms of its effects on disease processes and progression.

Antisense said no serious adverse events were reported.

The company said early indications of an immunomodulatory effect showed a downward trend during the treatment phase of certain immune cells, especially those expressing CD49d, and returned to starting levels post-dosing.

Antisense said the data showed improved strength compared to a six-month study that showed a significant mean reduction in upper body muscle strength and the data safety monitoring board recommended continuation of the trial.
Antisense chief executive officer Mark Diamond said that “given that there is currently no effective treatment for non-ambulant DMD patients, we are particularly encouraged by the preliminary data from the first six patients in this trial, which suggests a positive drug effect and may also demonstrate a meaningful slowing of disease progression compared to what might otherwise have been expected”.

“We expect this preliminary data to assist us in our planned regulatory interactions on the design and conduct of the phase IIb clinical trial that should allow examination of dosages of 25mg and higher to determine the optimal dosage,” Mr Diamond said.

Antisense said that ATL1102 was being developed as a novel treatment for the inflammation that exacerbates muscle fibre damage in Duchenne muscular dystrophy patients, currently treated with corticosteroids, which had a range of serious side effects when used for a prolonged period as required in Duchenne muscular dystrophy.

The company said it was the first occasion ATL1102 has been dosed for an extended duration, six months, in this patient population of non-ambulant Duchenne muscular dystrophy patients, so it expected the safety observations would be important support for the proposed longer dosing in a phase IIb trial.

Antisense said the progress of the final three patients, due to complete dosing in November, “appears consistent with this view that the drug is showing activity”.

The company said that a 2016 publication evaluated disease progression in non-ambulant boys over a six-month period, where a significant mean reduction in upper body muscle strength of the subjects was observed.

Antisense said that “by comparison, the data on the first six patients completing dosing in the ATL1102 trial, shows a distinct improvement in these strength parameters over the losses noted in the … publication”.

The company said it was further analyzing the preliminary trial data to confirm the level and rate of response to therapy within the trial to date.

Antisense climbed as much as 100 percent to 9.4 cents before closing up three cents or 63.8 percent to 7.7 cents with 36.8 million shares traded.