



MD research funded

EACH year, at least three boys born in WA will develop a devastating form of muscular dystrophy known as Duchenne (DMD).

Due to a faulty gene message, their muscles will gradually weaken so that eventually they can no longer walk, or even breathe, and most of them will die before they are twenty.

Young Garry Ellis, whose family has farmed at Bindi Bindi for four generations, was one of those boys.

Garry was only 16 years old when he died, but many years later his older brother, Agricultural Region MLC Brian Ellis, has made sure that his death was not in vain.

After attending a briefing on some breakthrough research which had been successful with DMD-affected mice, Brian Ellis and a parliamentary colleague met with Health Minister Kim Hames to secure special funding for human trials.

As a result, the State Government provided \$647,000 to test the revolutionary treatment.

In a process called 'exon skipping' an international team, including Prof Steve Wilton and Prof Sue Fletcher from the University of WA, will use 'molecular scalpels' to reprogram the body's machinery and snip out the disease-causing part of the gene message.

Their idea is similar to the way to which a 'patch' is used to remedy a computer software glitch, or like a band-aid which sits over the fault and tricks the body into skipping over the bad bit.

The molecular 'patch' replaces a missing protein which has not been built up because of the genetic mistake in the affected bodies.

The protein is essential to keep humans walking and breathing, because it acts like a molecular shock absorber, and strengthens and stabilises muscle fibres.

Mr Ellis said that it had been heart-breaking to watch his younger brother's health and independence decline.

Garry was in a wheelchair by the age of 12,

and had constant trips to Perth for medical appointments.

"Like most country people who are caring for an increasingly dependent family member, eventually we had the added strain of having to place Gary in residential care in the city," Mr Ellis said.

Perth mother Lesley Murphy, whose 21 year old son Conor is battling DMD and hopes to take part in the trial, said it takes courage to talk about these things.

"I really admire Brian for doing this, and for taking our cause to heart. The support of people like him is what keeps us going," Mrs Murphy said.

Lesley and Conor are planning a regional educational tour to make communities aware of DMD and the ongoing funding needs.

In the meantime, the Perth human clinical trial will be conducted in conjunction with WA Health by the Australian Neuromuscular Research Institute and the Muscular Dystrophy Association of WA.

Mr Ellis said the technique also has enormous implications for a variety of conditions including cancer, asthma, Alzheimers and diabetes.



Trial: Perth mother Lesley Murphy, whose 21 year old son Conor is battling a devastating form of muscular dystrophy hopes to take part in the revolutionary trial to treat the disease.



Age 12: Gary Ellis on the farm at Bindi Bindi was in a wheelchair by age 12.