AviadoBio and Taysha Gene Therapy announcements provide hope for Rett Syndrome.

I have previously written several articles on Gene Therapy with particular reference to Rett Syndrome and include links to these articles below that can otherwise be accessed via the RSAA website (https://rettaustralia.org.au/). This short article reports recent events which may influence future gene therapy for Rett Syndrome.

One of the companies which has pioneered gene therapy is Novartis; unfortunately, the company has decided to abandon its interest in Rett Syndrome but a recent announcement by the Australian Treasurer, Josh Frydenberg, is nevertheless of considerable interest to the Rett community. Novartis has developed a successful gene therapy approach to treat spinal muscular dystrophy (SMA). Like Rett Syndrome, SMA is a neurological disease caused by a mutation in a single gene that results in progressive muscle weakness, paralysis and premature death in young children. The gene to correct SMA is delivered by Adeno-associated Virus (AAV) in a pharmaceutical composition named Zolgensma.

However, the cost of Zolgensma is $2.6M per treatment thus placing the therapy beyond the means of most families. Consequently, the announcement by Josh Frydenberg that Zolgensma would be included in the Pharmaceutical Benefits Scheme means that the cost of therapy in Australia will be considerably lower, making the therapy available to most families.

The RSAA Committee is aware that future gene therapy for Rett Syndrome (see below) may be equally expensive and is prepared, in the future, to do everything in its power to lobby and advocate on behalf of Rett families in Australia to pursue a similar deal with the Pharmaceutical Benefits Scheme.

Readers of the RSAA Newsletter will be aware of an upcoming gene therapy clinical trial for Rett in Canada performed by Taysha, a company based in the USA. Taysha will also use AAV to deliver a modified form of the MeCP2 gene, as reported in a previous article on the RSAA website (https://rettaustralia.org.au/blog/a-game-changer-for-gene-therapy-for-rett-syndrome/). As this trial will focus on adults with Rett Syndrome, a recent announcement from another US company, AviadoBio, that they plan to develop gene therapy for early-onset (adult) dementia is good news for the Rett community, because the more we learn about the delivery of AAV to the human brain the more likely we are to develop a cure for Rett Syndrome.

This is an exciting time for AAV-based gene therapy projects, not least the upcoming Taysha trial to treat adults with Rett Syndrome. Our fervent hope is that this trial will provide sufficiently encouraging results to ensure that research continues with the ultimate goal of a cure for Rett.

Thanks to Tony Cagliuso, Gary Grocott and Claude Buda for helpful comments.
Eric Gowans (eric.gowans@adelaide.edu.au)
14 May 2022

Links to previous articles;
https://rettau.b-cdn.net/wp-content/uploads/2021/06/RSAA-rAAV-2-1.pdf
(link to the previous article on AAV)
https://rettau.b-cdn.net/wp-content/uploads/2021/08/AAV-progress-2.pdf
(link to an article on the progress of gene therapy to treat Rett)