Spinal Muscular Atrophy
AUSTRALIA INC.

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PBAC Decision- 20th April 2018

Spinal Muscular Atrophy Australia Inc. (SMA Australia) are extremely excited to advise the SMA community that the Pharmaceutical Benefits Advisory Committee (PBAC) at their most recent March meeting, has given a 'positive' recommendation for Spinraza (nusinursen) to be listed in the Pharmaceutical Benefits Scheme (PBS) for treatment of paediatric patients (treatment initiation in pateints18 years or younger). This includes Type 1, 2 and 3(a) - those under the age of 18 years and who have had onset of signs and symptoms consistent with SMA prior to 3 years of age.

This announcement by the PBAC, is a phenomenal outcome, especially as Biogen was asked to resubmit to the March meeting for SMA Type 1 only. When SMA Australia representatives were invited to the stakeholder meeting in January we highlighted the unmet need for urgent cases outside Type 1 and we asked the PBAC to consider those families as well. We would like to acknowledge the PBAC for inviting our community to the meeting and for listening to our voices that day on behalf of the whole community. It is great that they addressed our concerns and listed Spinraza for the broader paediatric population. Thank you!

Now that the PBAC has recommended Spinraza be listed on the PBS, we eagerly await Minister Hunt's announcement of this listing date. In the interim, from the 1st May 2018, Biogen will be making Spinraza available free of charge on a compassionate basis for those patients that fulfil the PBS criteria until the secured government funding of Spinraza commences. We thank Biogen for addressing the high unmet need for families here in Australia, and their continued effort in making the treatment available as fast as possible to the whole community. While Biogen has committed to the date of May 1, individual hospitals need to have made the necessary preparations before they can actually start administering to patients. Such preparations include hospital applications and approvals, staff resourcing and training. SMA Australia encourages you all to contact your clinician in your state to discuss and make arrangements regarding your treatment.

Disappointingly, the treatment needs of the SMA population over the age of 18 remain unmet. SMA Australia realise this part of our community will be devastated. Due to the infancy of the drug, and lack of robust clinical data, the PBAC was unable to assess the extent of the meaningful benefit the drug may have on the adult population.

SMA Australia will continue to work closely with the PBAC to identify what further information is required for the adult population and we will be calling on you in the future to assist us in gathering this information. We are also aware that Biogen will need community input for information they may require for any future PBAC submissions they may make. In the coming weeks we will be holding a webinar for the adult population to better represent what your needs are. We encourage the adult population to contact us so we are able to better present your case to the government.

If you aren't connected with us please register via the patient registry which is located http://smaaustralia.org.au/support-services/sma-patient-registry

Your assistance in these two matters will assist SMA Australia and add weight to continued discussions with the PBAC. We are available for further discussions as to the next steps and can be contacted on 03 9796 5744.

Kind regards,