Risdiplam: Adis Evaluation

Clinical Considerations

- First oral drug approved for the treatment of SMA
- Increases the production of full-length SMN protein as an *SMN2* pre-mRNA splicing modifier
- Improves motor function in patients with SMA types 1, 2 or 3, with benefits sustained for up to 2 years of treatment
- Generally well tolerated, with a favourable benefit to risk balance

Plain Language Summary

Background and rationale

- Patients with spinal muscular atrophy (SMA) often have insufficient levels of survival motor neuron (SMN) protein due to a defect in the *SMN1* gene.
- The *SMN2* gene is also able to produce some SMN protein, but not to the amount required to maintain adequate muscle function and form.
- Risdiplam (Evrysdi®) is a drug that targets *SMN2* to improve the production of viable SMN protein and the first oral medication approved for the treatment of SMA.

Clinical findings

- In the FIREFISH and SUNFISH clinical trials, risdiplam improved motor symptoms in patients of all ages for up to 2 years of treatment.
- Risdiplam was generally well tolerated, with a favourable benefit to risk balance.

Conclusion

As an orally administered treatment, risdiplam provides a convenient and useful treatment option across a broad range of patient ages and subtypes of SMA.