

Krabbe's disease and Orphan Status

How does a small British Pharmaceutical company become focussed on Krabbe's Leukodystrophy?

In 2004, the London Neurologist Dr. Richard Orrell and I were discussing curious instances of reversal of axonal conduction block in a patient with CIDP (which resembles a chronic form of the Guillain-Barre syndrome). She (and a number of multiple sclerosis patients before her), had been taking a hyper-immune goat serum product, self-administered by injection every four days. She had gained little benefit from cytotoxic, high dose steroid, pooled ivIG and plasma exchange treat-

ments over many years. A sodium channel effect seemed likely.

Richard told me of an adult male patient with the exceedingly rare adult form of Krabbe's disease. Informed consent was obtained and the patient remains on the medication as an MHRA "special", along with the CIDP patient, to this day. Uncontrolled observations of the responses of a total of four Krabbe's patients, led to an application for a TGA Orphan Status designation, granted shortly thereafter. This program was initiated by the FDA to encourage research into Motor Neuron Disease and other rare, "abandoned" conditions. Orphan status can lead to major advantages with respect to receipt of specialised R & D advice, assistance with aspects of the Marketing Authorisation application process and a

degree of product exclusivity.

We are now about to apply to embark on formal double-blind testing of the medication in Krabbe's disease. The extreme rarity of the condition presents problems, but the modern "N of 1" model, where a patient acts as their own control in a series of randomised "crossovers", is considered the best method. A multi-centre approach should provide appropriate recruitment levels. Only appropriately monitored and audited trialing will determine whether there is a useful palliative effect here, but it is undoubtedly true that we would not have got this far so quickly without the Orphan status. ■

FURTHER INFORMATION:

[Dr. Bryan Youl MD FRCP](#), Clinical Director
[Daval International Limited](#).



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