



In this report we demonstrate the criteria required to ensure a successful home treatment programme and describe our experience thus far” Finnigan et al (2017).

Abstract:

Enzyme replacement therapy is the only available treatment for Mucopolysaccharidosis type IVA (MPS IVA, Morquio syndrome). The treatment is lengthy and invasive involving weekly intravenous infusions of 4-5 h. This can cause significant disruption to normal family life so the provision of a safe and effective homecare service is essential. In order to deliver a safe service, robust standards must be in place; this includes appropriately trained members of homecare staff, detailed management for infusion related reactions (IRR) and appropriate venous access. In this report we demonstrate the criteria required to ensure a successful home treatment programme and describe our experience thus far.

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Reference:

Finnigan, N., Roberts, J., Mercer, J. and Jones, S.A. (2017) Home infusion with Elosulfase alpha (VimizimR) in a UK Paediatric setting. *Molecular Genetics and Metabolism Reports*. 14,



p.15-18. eCollection.

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