UK SMA Patient Registry

www.treat-nmd.org/registry

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UK SMA Patient Registry

- Patient driven online registry
- Launched in 2008
- Funded by SMA Support UK
- Over 400 people have signed up to the registry
- Similar numbers of people registering each year since 2009
What information is collected

Mandatory data items:
- Personal Data
- Clinical Diagnosis
- Genetic test result
- Ambulation
- Best motor function achieved
- Wheelchair use
- Scoliosis surgery
- Gastric/nasal tube
- Participation in a clinical trial

Highly encouraged data items:
- Ventilation
- Details of pulmonary function
- Positive family history
- SMA classification (type I, II, III)
- Molecular data

• The same list if information (genetic results and clinical data) collected by all SMA registries that are part of the TREAT-NMD network

• This can be very helpful for:
  – finding where the patients are
  – help with recruiting patients for clinical trials
TREAT-NMD Patient Registries

• Benefits to patients:
  – Information on standards of care and new research developments
  – Not being left behind as clinical trials develop
  – A link to the research community

• Benefits to researchers and industry
  – Standardized genetic and clinical data
  – Helpful for clinical trial planning:
    • Finding out where eligible patients are
    • Recruitment of patients into clinical trials
TREAT-NMD affiliated SMA Registries in 2013

>5,000 patients
Global network of over 35 national SMA patient registries

The design, setup and utilisation of the TREAT-NMD national SMA patient registries described in the paper: "Mapping the differences in care for 5000 Spinal Muscular Atrophy patients, a survey of 24 national registries in North America, Australasia and Europe", Bladen C.L. et al, Journal of Neurology 2013, October
How is the UK SMA Patient Registry used?

Use by Industry

- Trophos study
- Increase in SMA clinical trials and interest in utilisation of the registry

Use by academia and regulators

- Medicines and Healthcare products Regulatory Agency (MHRA) questionnaire
- Imagining Futures study (funded by ESRC, Warwick University)
What’s new?

• Registry upgrade
• Communication
• SMA REACH UK collaboration
UK SMA Registry - demonstration

https://www.treat-nmd.org.uk/registry/
Questions...?

Thank you!

Further information:
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