



The International Pediatric MS Study Group - IPMSSG

EMSP Oslo- May 2016

Rogier Hintzen neurologist





MOTIVATION FOR FOCUS ON PEDIATRIC MS

DUTCH/ROTTERDAM SITUATION

- -working on aggressive variants/BMT
- -(desparate) questions by 3P (patients, parents, professionals): gap in knowledge, limited pool for research
- -growing international attention
- -scientific: unique window into MS risk factors

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WHY IPMSSG?

Rare disease in children

No approved therapies or standardised clinical practices

Limited pool for research

Only network to serve the global pediatric MS clinical and research community

Pediatric MS offers a unique and vitally important window into MS risk factors, genetics and immunology



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Vision

... "to optimize worldwide care, education and research in pediatric multiple sclerosis (MS) and other acquired inflammatory demyelinating disorders of the central nervous system." (IPMSSG Charter, 2013)

Mission

To improve the care of children with multiple sclerosis and acquired demyelinating diseases worldwide, promoting clinical initiatives, education and research.

Timeline and Key Activities



- Inaugural meeting
- Consensus publications
- Decision to formalize international collaboration

- International Pediatric
 MS Research Meeting
- Pilot project
- Outreach

Forming 2004-2007 **Growing 2007-** 2009

Initiating 2009-2011

- Charter with guiding principles for collaboration
- 100+ members

Key Activities





- IPMSSG Clinical Trials Summit
- Research Writing Group

- Implementing the strategic plan
- Establishing the new infrastructure

Guiding 2011-2013

Evolving 2013-2015

2015 +





- Protocol reviews
- Charter revision
- Boston 2014





2004: meeting of < 20 clinicians



- Consensus:
 - What is MS?
 - How do we manage?

2014: > 135 members from 36 countries

- Charter and Strategy
- Clinical Care
- Clinical Trial
- Education and Outreach
- Research





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What Impact Has It Had?

- Better patient care for all children with MS
- Early Diagnosis (definitions)
- Treatment; given a choice (consensus)
- Evidence- treatment trials (summit)
 - Drug companies want/need our involvement
- Addressing the child's needs cognition
- National networks USA, UK, Holland, Canada, Poland, Norway
 - Minimum standard of care for all children

Achieved

Clinical Care

2007 Publication of 1st consensus publication – **Neurology** supplement

2011 Consensus of treatment

2013 Revision of definitions

Clinical Trials

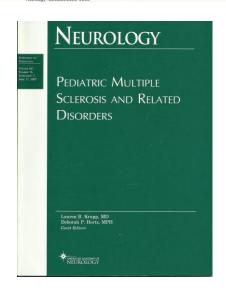
2012 IPMSSG Therapeutic Summit Workshop with experts, agencies and pharma

Clinical Trials Task Force

Engagement with drug companies, FDA, EMA

Tanaja Chania, MD Mar Tanka, MD Market Market, MD Mar

Conclusions: Consensus points for conduct of clinical trials in the rare disease pediatric MS were identified amongst a panel of academic experts, informed by regulatory and industry stakeholders.





Abstract

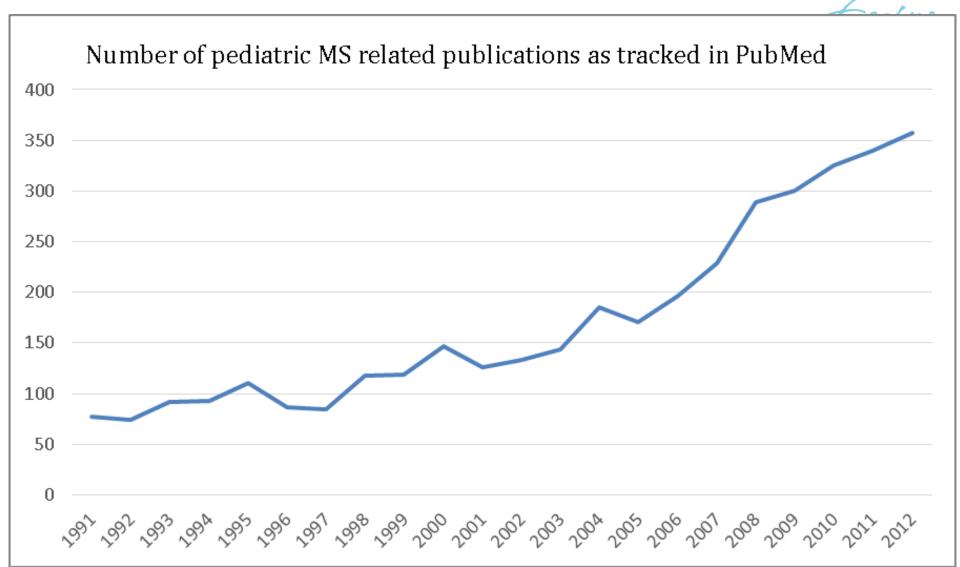
of these new and existing agents in the management of pediatric NS has yet to be defined. Pediatric investigation plans are now required by the Food and Drug Administration and European Medicines Agency for approval of new biological agents, providing an important copportunity to gather much-needed data for clinicians caring for children and adelescents with MS. However, challenges include the small number of pasients, and the need for reflicient, yet comprehensive study designs incorporating factors necessary to inform the clinical care of children with PKS. The elected Stearing committee modifying the register of the properties of t

Pediatric multiple sclerosis and pediatric demyelinating disorders: global update and future directions – Neurology supplement 2016



- 1. Perspectives and concerns from patients and their families.
- 2. Consensus definitions pediatric multiple sclerosis and other demyelinating disorders.
- 3. Immunopathophysiology and biomarkers.
- 4. Environmental and genetic factors in pediatric inflammatory demyelinating diseases.
- 5. Differential diagnoses.
- 6. Acute disseminated encephalomyelitis.
- 7. Transverse myelitis.
- 8. Optic neuritis.
- 9. Neuromyelitis optica spectrum.
- 10. Acquired demyelinating syndrome and multiple sclerosis.
- 11. Clinical features and outcome.
- 12. Cognition and mood.
- 13. MRI-features.
- 14. First-line treatment and general management.
- 15. Pediatric multiple sclerosis: Escalation and emerging treatments.
- 16. Challenges and future







Management-care research
Information training



And fun! +patients voice...

