Volumetric MRI sensitively measures plexiform neurofibroma growth in NF1

Percent change in plexiform neurofibroma volume (A) and body weight (B) over time in 49 patients with neurofibromatosis type 1 and plexiform neurofibroma.

Dombi et al. used automated volumetric MRI analysis to measure plexiform neurofibroma growth in neurofibromatosis type 1 (NF1). Plexiform neurofibroma growth varied among patients, but was constant within patients. Younger patients had the most rapid plexiform neurofibroma growth rate. Volume increase of plexiform neurofibroma is a meaningful trial endpoint, and age stratification for clinical trials for plexiform neurofibroma should be considered.

see page 643

Commentary by Patrick Y. Wen, MD; and Jan Drappatz, MD

NF1 is an autosomal dominant disorder with an incidence of 1:3,000. Approximately 20 to 44% of patients with NF1 develop peripheral nerve sheath tumors called plexiform neurofibromas. These are a major source of morbidity, producing disfigurement, pain, and impairment of neurologic function. Approximately 5% of cases also undergo transformation into malignant peripheral nerve sheath tumors. Currently there is no effective therapy for these tumors with the exception of surgery in a subset of patients.

The development of more effective medical therapies for plexiform neurofibromas in patients with NF1 has been limited by our poor understanding of the natural history of these tumors, their variable rate of

growth, and their large and complex shapes, limiting the usefulness of standard measurements of tumor response such as the one-dimensional Response Evaluation Criteria in Solid Tumors (RECIST) and the two-dimensional World Health Organization (WHO) criteria. Dombi et al. used automated volumetric MRI to effectively document the growth of plexiform neurofibromas over time. They showed that the rate of growth of plexiform neurofibromas was variable and that the highest growth rate occurred in the youngest patients. The ability to monitor plexiform neurofibromas closely greatly facilitates the conduct of clinical trials of novel therapeutic agents for plexiform neurofibromas. It also allows patients to un-

dergo therapeutic intervention earlier if an increase in growth rate suggestive of malignant degeneration occurs. The utility of volumetric MRI in monitoring tumor response was validated in a recent phase I study of the antifibrotic drug pirfenidone in plexiform neurofibromas.¹ Automated volumetric MRI is likely to find widespread acceptance in the monitoring of plexiform neurofibromas and greatly help in the search for more effective therapies for this disabling condition.

Reference

1. Babovic-Vuksanovic D, Widemann BC, Dombi E, et al. Phase I trial of pirfenidone in children with neurofibromatosis type 1 and plexiform neurofibromas. *Neurology* 2007 (in press).

see page 643

Neurology®

**February 27 Highlight and Commentary: Volumetric MRI sensitively measures
plexiform neurofibroma growth in NF1**

Neurology 2007;68;627

DOI 10.1212/01.wnl.0000257907.93267.52

This information is current as of February 26, 2007

**Updated Information &
Services**

including high resolution figures, can be found at:
<http://n.neurology.org/content/68/9/627.full>

Permissions & Licensing

Information about reproducing this article in parts (figures,tables) or in
its entirety can be found online at:
http://www.neurology.org/about/about_the_journal#permissions

Reprints

Information about ordering reprints can be found online:
<http://n.neurology.org/subscribers/advertise>

Neurology® is the official journal of the American Academy of Neurology. Published continuously since 1951, it is now a weekly with 48 issues per year. Copyright . All rights reserved. Print ISSN: 0028-3878. Online ISSN: 1526-632X.