

# The Linking Hand

Across Australia and NZ

September/October 2004



## Our Motto

A life is not measured by the number of days, months  
or years that it is lived,  
but by the effect that life has on other people who touch it.  
It is not measured by the length of its days or the height  
of its achievements,  
but by the breadth of its influence.

Anonymous

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## TKT Remains Committed to MPS II

Together with the support of the MPS Societies, patients, and families, Transkaryotic Therapies, Inc. (TKT) was able to successfully enrol a pivotal trial evaluating its enzyme replacement therapy for the treatment of MPS II. With 96 patients, this is the largest study ever conducted for a lysosomal storage disorder. If the results are positive, TKT plans to seek marketing approval in both the United States and Europe in the second half of 2005, followed by other regions of the world.

In addition, TKT's researchers have been working to understand how to administer treatments to specifically target the central nervous system of certain disorders. TKT has been evaluating new approaches and if the results support it, the company hopes to conduct a trial to directly administer the enzyme into the central nervous system in MPS II patients in 2006, at the earliest. TKT believes success with this program may enable the company to develop treatments for other lysosomal storage disorders with significant CNS involvement, including Sanfilippo syndrome.



Jack Peacock (MPSII) 14mths, at MPS Society Conference Melbourne 2004, BBQ lunch