

Patient Advocacy Organization EMPOWER Q&A

What are the EMPOWER results?

EMPOWER did not meet its primary endpoint, a joint rank analysis of function and survival, and no efficacy was seen in the individual components of function or survival. The trial also failed to show efficacy in its key secondary endpoints. Additional analyses of multiple subpopulations failed to demonstrate any efficacy among these groups.

What is EMPOWER?

EMPOWER was a randomized, double-blind, placebo-controlled Phase 3 trial which enrolled 943 people with ALS at 81 sites in 11 countries. Patients were randomized on a one-to-one basis to receive either dexamipexole or placebo. The primary endpoint was a joint rank analysis of function and survival, known as the Combined Assessment of Function and Survival (CAFS). In addition to CAFS, the trial individually evaluated functional decline, survival and respiratory decline among other measures.

The trial enrolled 943 people with either sporadic or familial ALS and symptom onset within 24 months of randomization. Patients from 81 study sites in 11 countries (Australia, Belgium, Canada, France, Germany, Ireland, The Netherlands, Spain, Sweden, United Kingdom, and the United States) were followed for up to 18 months.

What happens now?

We share the disappointment of the ALS community, who had hoped that dexamipexole would offer a meaningful new treatment option. Nevertheless, the EMPOWER trial represents a significant contribution to ALS research as it created a robust research database and established a new endpoint for ALS trials.

Based on these data, Biogen Idec will discontinue further development of dexamipexole in ALS and work with investigators to appropriately and sensitively end the ENVISION extension trial.

Biogen Idec intends to present detailed EMPOWER results at a future medical conference.

Is Biogen Idec going to continue its work in ALS?

Biogen Idec is committed to advancing ALS science and is working with researchers around the world to further understand the causes of ALS and find potential new treatments for people with ALS. The company has many ongoing R&D programs and active collaborations, including:

- Investigation of multiple compounds in ALS.
- Research collaboration with Duke University and HudsonAlpha Institute to sequence the genes of up to 1,000 patients with ALS in an effort to gain a deeper understanding about the fundamental genetic causes of the disease.

- Research agreements with several academic institutions with the shared goal of advancing our understanding of ALS.
- Research consortium in collaboration with several leading academic research centers to identify new approaches to treating ALS. Each of the centers involved in this consortium brings different scientific and technical expertise and a shared goal of improving the understanding of ALS from a basic science perspective.
- Multiyear funding commitment to the University of Massachusetts Medical School ALS Champion Fund. The funding will support ALS awareness efforts and basic and clinical science research into potential treatments.

Is there someone I can talk to for more information about dexamipexole, EMPOWER or Biogen Idec's work in ALS?

Information about EMPOWER, dexamipexole and Biogen Idec's commitment to ALS is available by calling 877-312-9676 or 919-993-7311 in the U.S. or +44 1932 824112 outside of the U.S.