

July 31, 2016 via email

[PDGrpLdrs] Update on nilotinib and MJFF webinar on August 2nd

PD support group leaders,

I first started hearing about the leukemia drug nilotinib back in 2013. You can find a post here:

www.brainsupportnetwork.org/early-research-cancer-drug-nilotinib/

In 2013, Georgetown published some basic research that showed that nilotinib getting rid of alpha-synuclein in the brains of transgenic mice with Parkinson's. A Georgetown press release mentioned that they wanted to do phase II studies in PD, LBD, MSA, and PSP -- a surprising combination since PSP is not an alpha-synuclein disorder.

In October 2015, Georgetown announced results of a phase I clinical trial of nilotinib in 12 patients for a 6-month period. Unfortunately there was a mix of diagnoses -- 5 had a DLB (dementia with Lewy bodies), 3 had PD-MCI (PD-mild cognitive impairment), 2 had PDD (Parkinson's disease with dementia), one had PD plus MCI, and one had PD. Phase I studies are safety studies so I found it confusing that the researchers claimed the "drug improved cognition, motor skills, and non-motor function" in the 11 patients who completed the study. (One had to drop out due to a serious side effect.) The study size was very small and there was no placebo-control. Also, the cancer drug costs several thousand dollars per month.

People are still talking about nilotinib, especially in the PD and LBD communities. The Georgetown team's phase I results were finally published in July 2016. You can read the results here:

Nilotinib Effects in Parkinson's disease and Dementia with Lewy bodies

content.iospress.com/articles/journal-of-parkinsons-disease/jpd160867

Along with the Georgetown results, the journal published an accompanying editorial titled "Nilotinib - Differentiating the Hope from the Hype." The authors of the editorial said: "We debate the safety of Nilotinib and the reported efficacy signals. We emphasize that due to the small sample size, and lack of a control group, it is impossible to rule out a placebo effect." You can read that editorial here:

Nilotinib - Differentiating the Hope from the Hype

content.iospress.com/download/journal-of-parkinsons-disease/jpd160904?id=journal-of-parkinsons-disease%2Fjpd160904

If nilotinib is of interest to you, I'd suggest checking out this Michael J. Fox Foundation overview of the scientific rationale behind nilotinib:

www.michaeljfox.org/foundation/news-detail.php?nilotinib-update-where-we-stand-with-cancer-drug-for-parkinson

Or, if you still want more, the Michael J. Fox Foundation will be having a webinar on Tuesday, August 2nd, from 9 to 10am CA time about nilotinib as a possible treatment for PD.

You can register here:

www.michaeljfox.org/understanding-parkinsons/webinar-registration.php?id=11&e=1224270

The panelists "will discuss the scientific rationale behind nilotinib for Parkinson's, findings from recent research and next steps in the study of this drug. Q & A with the audience will follow the discussion."

Robin