

Novartis ends the VIBRANT-HD study and will not continue the program with Bramaplan. Still the study brings us some very good news as the drug did reduce the levels of mutant (sick) huntingtin in the spinal fluid collected from the participants. This is groundbreaking achievement as the drug is administered orally (through the mouth). Nevertheless, reducing huntingtin in a safe way seems to be a challenging process.

In August the ongoing Phase II study with Bramaplan was paused because some of the participants had negative side-effects of the treatment. Since then Novartis has spent time to understand what happened and why it happened and today they announced that the program will be stopped. The analyzes of the data collected from the trial participants showed that there were several negative sideeffects. Many, although not all, participants had injuries to nervecells outside the brain and spinal cord, so-called peripheral neuropathy. The levels of NfL (Neurofilament light) was increased and this is regarded as a sign of stress in the brain/nervesystem. A third negative sign that was found in the participants was an increase of fluid in parts of the brain.

After having studied all the results carefully and considered options to change the dosing etc, the Novartis team decided that they will end the Bramaplan program in HD. This is because its seen to be too many negative things happening although the levels of sick huntingtin is lowered.

-This time I was prepared for a negative result like this, says Astri Arnesen, president of EHA. It's of course sad and disappointing that Bramaplan is not safe to take, nevertheless it is a great positive thing coming out of this study: a drug taken through the mouth did lower the levels of huntingtin. That is a major step forward. Luckily we have a lot of other promising projects ongoing so even with this «horse» out of the race – we still are very much into the race with several other good ones!