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A challenge to expedite Genervon's new ASL drug

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Nicholas Grillo, 54 year-old with ALS.

(Medical Xpress)—The Ice Bucket Challenge to raise awareness for Amyotrophic lateral sclerosis (ALS) went viral on social media last summer. Over 1.2 million videos were posted on Facebook alone. The difficulty in treating a disease like ALS is highlighted by the fact that the only drug available for it, Riluzole, extends life expectancy by just a couple months. A new challenge was just issued to the FDA to make another drug available to sufferers of ALS. Specifically, a petition on Change.org by Nicholas Grillo for access to Genervon's GM6 drug has

just acheived a critical mass of over 100,000 signatures.

Our purpose here is not to plug Charge.org, but rather to inform. Don't get me wrong they have some great causes to stand up for—no brainers like a getting a few square inches of extra real estate to relieve helpless farm animals crammed into cages, or similarly win freedom for convicts still languishing in jail because of red tape despite being legally cleared of all wrongdoing. After signing these humanitarian causes however, one tends to get presented with additional, less clear-cut, concondrums of a more social-political nature like climate or gender issues.

The petition for Nicholas fails squarely in the first class of petitions—a no-brainer. Genervon's studies and trials have shown clear indications of efficacy for GM6. They are also willing to make it available, and insurance providers appear to be on board. But rather than just take our word for it, we might take a look at some of Genervon's open available patents for clues as to what exactly GM6 might be, and what it might actually do.

It appears that GM6 is the name for a whole class of compunds that are basically just short peptide strings of amino acids, perhaps with some specific modications. The one that has shown promise for ALS (GM604) contains a sequence identical to a particular active site on an endogenous trophic factor known as Motoneuronotrophic factor (MNTF). While many neuro-aware readers would have heard about some of the usual neurotrophic factor suspects—namely NGF, BDNF, CNTF—this MNTF may be a new one. A Google search, for "MNTF Receptor" had this to say: "Did you mean: cntf receptor?".

None-the-less MNTF seems to be a viable therapeutic target. Several of Genervon's embodiments and "analogs thereof" alluded to in their IP claims indicate six or seven amino-acid length peptides variously given as analogs of "WMLSAFS" and "FSRYAR" domains. The letters here

being the abbreviated name of the individual amino acids therein. Of note, injected GM604 clearly penetrates the Blood Brain Barrier and is capable of inducing anti-inflammatory or anti-apoptotic pathways. Less specifically, there are claims of GM604 controlling some 4000 genes and modulating 30 ALS related genes. Fortunately there are also some more specific incidentals given.

In particular, GM604 modulated the ALS-associated biomarker SOD1 (superoxide dismutase) both in blood plasma and in the CSF. Additionally it affected several so-called target/prognostic biomarkers like TAU, Compliment C3, TDP43 and Cystatin C. Perhaps more importantly, in a compassionate-use trial the drug was found to significantly alter the course of disease progression. ALS symptomatology can be difficult to quantify and one of the indicators used for this is swallowing volume. One long term patient doubled his swallowing volume after just two weeks of treatment.

Last spring there was a poignant case very similar to this one involving a critically ill seven-year-old named Joshua Hardy. Chimerix, the makers of a potentially life-saving drug called brincidofovir, initially refused to furnish the drug for him, despite his parents willingness to pay for it, because he did not fit into their trial plan. A massive twitter storm pressured them into providing it and Josh was then quickly able to defeat the hospital-acquired adenovirus which threatened his life. This instance was another no-brainer: Josh could not continue taking the standard drug, cidofovir, because it had compromised his kidney function following the immunosurpression phase of his bone marrow transplant for his cancer. Brindcidofovir was clearly the answer for him, in foresight and in hindsight.

Unlike the Chimerix case, Genervon is on the side of reason and compassion here, and hopefully enough readers of Nicholas Grillo's case will be too. Without some kind of fast track approval the wait could be

three years for GM6—three years ALS patients like him clearly do not have. In general, a short peptide string would not first appear to be that complicated of a beast. Absent the finer details, there could of course be more exotic modifications involved, like for example, functionalized sidechains or perhaps incorporation of D-form aminos.

Although we do not typically associate peptide based drugs with some esoteric biosynthesis process, nor an expensive or rare organic source, there are undoubtedly minor details that need to be considered in its procurement of which we, as outsiders, are not fully aware. Hopefully patients like Nicholas will soon be able to get what they need, even if it is not the perfect cure they seek.

More information: www.genervon.com/genervon/abou … pressreleasestxt.php

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