Mataró (Barcelona), October 11, 2021

Kelly Miettunen
Executive Director, ALD connect

Dear Ms Miettunen

On behalf of Minoryx Therapeutics I would like to provide you an overview on the recent interruption of dosing with leriglitazone on patients participating at the Advance Open Label Extension in the US.

This is because the FDA placed a clinical hold based on their assessment of the benefit-risk profile. In their view, there is a lack of sufficient evidence of effectiveness in the study Advance, while based on preclinical studies there is a potential carcinogenic risk. No human safety data has been raised as a concern by the FDA.

In order to resume the dosing of patients, FDA is requesting either the performance of preclinical carcinogenesis studies, or the justification of a favorable benefit-risk determination on the AMN population.

Even though we acknowledge that the study missed the primary endpoint and that there is a theoretical risk of carcinogenicity in humans, we believe that important clinical benefit was observed in pre-defined secondary endpoints, particularly on those related with progression of cerebral lesions and balance. We do not consider the benefit-risk balance of leriglitazone to have changed. Thus we believe that continuing the open label extension would be justified, while the additional preclinical data requested by FDA is generated in parallel.

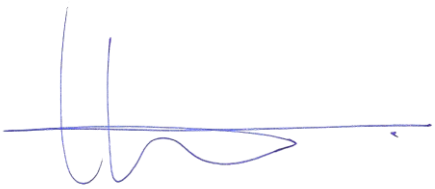
We are currently preparing a complete response for FDA to document our favorable benefit-risk profile by providing more information on the most relevant results. We hope that this will allow resuming the treatment for those patients that were in the Open Label Extension in the upcoming weeks.

Note that the Open Label Extension continues in Europe and EMA advised not to initiate yet preclinical carcinogenesis studies.

All the details on the interactions with FDA as well as relevant data have been shared with the study investigators. For patients affected by this situation we recommend to reach out to the clinical sites in case of any question or concern. As soon as we have feedback from FDA on our response, we will also immediately communicate it to them.

As a final note, let me reassure all the ALD community that we remain strongly committed to bringing a therapeutic solution for this terrible disease.

Sincerely yours,



Marc Martinell, PhD

CEO, Minoryx Therapeutics