



FOR IMMEDIATE PUBLICATION

NeuroVia Initiates Phase 1/2 Trial of NV1205 for Treating Childhood Cerebral Adrenoleukodystrophy (CCALD)

San Francisco, CA, July 25, 2018 – NeuroVia, Inc., a biopharmaceutical company focused on developing innovative therapies for rare, genetic neurological diseases, today announced the initiation of a Phase 1/2 clinical study of their lead drug candidate, NV1205, in patients with childhood cerebral adrenoleukodystrophy (CCALD). NV1205 is being developed to target the underlying metabolic defect in all phenotypes of X-linked adrenoleukodystrophy (X-ALD) patients by facilitating the metabolism of very long chain fatty acids (VLCFA) in the brain and adrenal tissue, which are the two main sites of the disease's pathology. X-ALD is a severe and often fatal neurodegenerative disease with limited treatment options and a significant need for innovation. NV1205 has the potential to significantly improve the current therapeutic landscape.

“Advancing our lead candidate NV1205 into clinical trials represents a key milestone in the company's development. The study initiation in Australia, Chile and the United Kingdom is just the first step in our global clinical development strategy,” said John Henderson, Chief Development Officer of NeuroVia. “We believe NV1205 has real potential to better serve the X-ALD community as a novel, pharmaceutical treatment for all X-ALD patients.”

The Phase 1/2 study (ClinicalTrials.gov Identifier: NCT03196765) is designed to assess the safety, tolerability and pharmacokinetics of increasing doses of NV1205 in patients diagnosed with CCALD and will be conducted in eight different countries – Argentina, Australia, Chile, Colombia, France, the Russian Federation, Ukraine and the United Kingdom. The trial will include an open label dose escalation study of multiple dose levels of NV1205 with a long-term treatment phase.

For more information and updates on the trial, please consult www.clinicaltrials.gov or <http://www.ccald.com>.

About NV1205

NeuroVia's lead compound, NV1205, is being developed as a novel therapeutic option for X-ALD patients of all phenotypes. NV1205 is a synthetic small molecule drug candidate that aims to restore metabolic activity within the cells by enabling them to process the detrimental, accumulating very long chain fatty acids (VLCFA) and avoid the resulting damage to neuronal and mitochondrial membranes altogether.

About X-ALD

X-linked adrenoleukodystrophy (X-ALD) is a rare, genetic disorder affecting 1 in 17,000 people worldwide that causes adrenal gland dysfunction and loss of the protective sheath that surrounds nerve fibers in the central nervous system. The childhood version progresses quickly and results in a severe neurodegenerative phenotype, which is often fatal within three to five years after onset of initial symptoms. The adult-onset version is characterized by numbness in the legs leading to difficulty or inability to walk and urinary and bowel incontinence. With no treatment options



currently available for symptomatic X-ALD patients, there exists an unmet medical need for a therapeutic benefit for X-ALD patients.

About NeuroVia

NeuroVia, Inc. is committed to addressing unmet medical needs in neurological diseases with the primary goal to arrest the onset of devastating neurological deficits associated with X-ALD. For more information, please visit: <http://www.neurovia-inc.com/>

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