PaxMedica Announces Positive Results from Phase 2 Trial of PAX-101 (IV suramin) in Children with Autism Spectrum Disorder (ASD)

- *PAX-101 demonstrated sustained improvements over placebo in the trial's primary and several secondary endpoints and a favorable safety and tolerability profile*
- *Results build upon data from a prior published pilot study, with new insights on the relationship between dose, efficacy and response over 14 weeks of treatment with this investigational drug for core symptoms of ASD*
- *The company is actively planning an IND submission in the U.S. and Clinical Trial Application (CTA) in Europe to prepare for the conduct of additional clinical trials*

WOODCLIFF LAKE, N.J., February 9, 2021 – PaxMedica, Inc. ("PaxMedica" or the "Company"), a biopharmaceutical company focused on the development and commercialization of innovative treatments for unmet needs in neurodevelopmental disorders, today announced positive topline data from its Phase 2 dose-ranging clinical trial evaluating PAX-101 (IV suramin), an investigational drug with a novel mechanism that the Company is developing as a potential treatment for the core symptoms of Autism Spectrum Disorder. With no FDA approved treatments available currently, PAX 101 has the potential to be the first drug approved for treatment of the core symptoms of ASD.

"This is an exciting step forward for the Autism community. The results from this clinical trial clearly show promise for advancing this novel treatment into the next phase of development," said Alice Mao, MD, Medical Director of Memorial Park Psychiatry Adult, Adolescent and Child Psychiatry in Houston, TX. "There is an enormous unmet need in treating the symptoms of moderate to severe ASD, which include both social and communication challenges as well as restrictive, repetitive behaviors. PAX-101 has the potential to demonstrate meaningful clinical improvements in this debilitating condition, which could positively impact the lives of many families."

The Phase 2 study was a dose-ranging, randomized, double-blind, placebo-controlled, multidose trial evaluating the safety and efficacy of PAX-101 in patients diagnosed with moderate to severe autism spectrum disorder. In the 14-week trial, patients were randomized 1:1:1 to receive 10mg/kg of PAX-101, 20mg/kg of PAX-101 or placebo infusions every 4 weeks. Infusions were administered at baseline, week 4 and week 8. There was a 6-week follow-up after the last dose of study medication.

The trial enrolled 52 patients from 6 sites across South Africa. The study population included a patient population with diverse ethnicity and the mean age for the study population was 8.43 (± SD 3.24). Forty-three (43) subjects completed the study, with 5 withdrawals due to COVID-19 lockdowns, 1 for an adverse event, and 2 for other reasons.

The primary endpoint of the study was the change between baseline and Week 14 in the Aberrant Behavior Checklist (ABC) composite score of core symptoms (ABC Core) including ABC-II (lethargy/social withdrawal), ABC-III (stereotypy) and ABC-V (inappropriate speech). Secondary outcome measures included ABC individual sub-scores, Clinical Global Impression of Improvement scale, adapted for autism (CGI-I), Autism Diagnostic Observation Scale, version 2 (ADOS-2) changes, Autism Treatment Evaluation Checklist (ATEC), and safety and tolerability.

PAX-101 demonstrated consistent clinical activity in this study, as evidenced by the achievement of marked and sustained improvement in several efficacy assessment measures, including the ABC Core,

CGI-I and ATEC, all compared to placebo over the study's 14-week duration. At Week 14, there was a mean 48% improvement from baseline in the ABC Core in patients on active treatment vs. 31% on placebo, with twice as many actively treated patients exhibiting a 70% or greater improvement vs. placebo. At Week 14, patients treated with PAX-101 also demonstrated a mean improvement from baseline in the CGI-I overall symptom severity score of 0.9 points versus 0.4 on placebo, representing a clinically meaningful change from baseline. Certain key subpopulations demonstrated even further improvements on these and other assessments. This trial was designed as a robust dose-ranging study to confirm and expand upon the initial data from a prior published single dose, single site, pilot study, but was not powered to demonstrate statistical significance across endpoints.

The safety and tolerability of suramin has been well characterized in the treatment of other illnesses. During this study, two different doses of PAX-101, administered monthly for 3 months, were shown to be safe and tolerable throughout the 14 weeks of treatment. The most common treatment-emergent adverse events in drug treated patients were rash, upper respiratory infection and vomiting. Most events were mild to moderate in severity and resolved with no intervention. There was one serious adverse event in a single patient on PAX-101 with multiple concomitant conditions that resolved without sequelae following acute treatment.

"We are extremely grateful to all of the trial participants, their families, and the investigators and trial site staff for helping us complete this important study," David Hough, MD, Chief Medical Officer of PaxMedica, commented. "The company is already working to develop plans for the next set of clinical trials for moderate to severe autism and engaging with FDA on the path to approval."

PaxMedica expects to submit a full analysis of the Phase 2 data for presentation at an upcoming medical conference and publication in a peer-reviewed scientific journal. The full analysis will include data from primary, secondary, and exploratory endpoints evaluated in the trial, safety and laboratory data, and an analysis of the pharmacokinetic data.

PaxMedica's mission is to change the therapeutic paradigm for ASD and other neurodevelopmental disorders. In addition to PAX-101, PaxMedica is developing PAX-102, a proprietary intranasal formulation of suramin for less severe forms of ASD as well as other neurodevelopmental disorders.

About PAX-101 (IV suramin)
PAX-101, an antipurinergic agent delivered as an IV infusion, has also been shown to have significant anti-inflammatory properties based on non-purine receptor pathways in multiple disease models. The mechanism of the drug's action in a condition like ASD is not fully understood and has been postulated to act through purinergic receptor blockade to reverse the effects of mitochondrial dysfunction, but also has been postulated to act through the reduction of neuroinflammation in this population.

About Autism Spectrum Disorder (ASD)
ASD refers to a group of complex neurodevelopmental disorders characterized by repetitive and characteristic patterns of behavior and difficulties with social communication and interaction. These symptoms are present from early childhood and affect daily functioning of individuals with ASD. The term "spectrum" refers to the wide range of symptoms, skills, and levels of disability in functioning that can occur in people with ASD. ASD occurs in every racial and ethnic group, and across all socioeconomic levels. Approximately 1 in 54 children in the U.S. is diagnosed with an autism spectrum disorder with boys being four times more likely to be diagnosed than girls. There are presently no FDA approved therapies for the core symptoms of autism.

About PaxMedica, Inc.
PaxMedica is a clinical-stage biopharmaceutical company focused on developing innovative treatments for unmet needs in neurodevelopmental disorders. Our lead programs are focused on two of the world's most challenging conditions – Autism Spectrum Disorder (ASD) and Fragile X-associated Tremor / Ataxia Syndrome (FXTAS). There are no FDA approved treatments for the core symptoms of ASD and no FDA approved treatments at all for FXTAS. For more information, visit https://www.paxmedica.com/.

Forward-Looking Statements
This press release contains certain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934 and Private Securities Litigation Reform Act, as amended, including those relating to the Company's product development, clinical and regulatory timelines, market opportunity, competitive position, possible or assumed future results of operations, business strategies, potential growth opportunities and other statements that are predictive in nature. These forward-looking statements are based on current expectations, estimates, forecasts and projections about the industry and markets in which we operate and management's current beliefs and assumptions.

These statements may be identified by the use of forward-looking expressions, including, but not limited to, "expect," "anticipate," "intend," "plan," "believe," "estimate," "potential, "predict," "project," "should," "would" and similar expressions and the negatives of those terms. These statements relate to future events or our financial performance and involve known and unknown risks, uncertainties, and other factors which may cause actual results, performance, or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. Such factors include those set forth in the Company's filings with the Securities and Exchange Commission. Prospective investors are cautioned not to place undue reliance on such forward-looking statements, which speak only as of the date of this press release. The Company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events or otherwise.

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