PAXMEDICA

Addressing Urgent Needs In Serious Neurologic Disorders

August 2023 • Nasdaq: PXMD

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Overview

PaxMedica is a clinical stage biopharmaceutical company focused on the development of anti-purinergic drug therapies (“APT”) for the treatment of disorders with intractable neurologic symptoms including Autism Spectrum Disorder (“ASD”).

PAX-101, an intravenous formulation of suramin, demonstrated a statistically significant and clinically meaningful change on the CGI-I scale in a Phase 2 clinical trial for ASD.

PAX-101 has demonstrated positive topline results in a phase 3 clinical trial for the treatment of Human African Trypanosomiasis (HAT), a rare and fatal tropical disease.

Investment Highlights

- PaxMedica is the only public company focused on developing treatments for the core symptoms of Autism Spectrum Disorder (ASD)
- Currently preparing for a clinical trial of PAX-101 in ASD
- Multiple catalysts expected through 2024
  - Planning a multi-centered clinical trial of PAX-101 in ASD
  - Preparing NDA filing for approval of PAX-101 for the treatment of Human African Trypanosomiasis (HAT), a rare and fatal tropical disease
    - Expect to file in 2024
  - Potential award of U.S. Rare Tropical Disease Priority Review Voucher (PRV)*
- Ample available capital for near-term needs
- Independent Board and management team of experienced industry entrepreneurs

* For full analysis see GAO Report GAO-20-251, as of January 31, 2020

Management Team Led by Experienced Pharma Entrepreneurs

Howard J. Weisman
Chief Executive Officer

Stephen Sheldon
Chief Operating/Financial Officer

Dr. David Hough
Chief Medical Officer

Buzz Woods
Corporate Communications

Strong Board of Directors

Howard J. Weisman

Zach Rome

Karen LaRochelle

Dr. John F. Coelho

Charles J. Casamento

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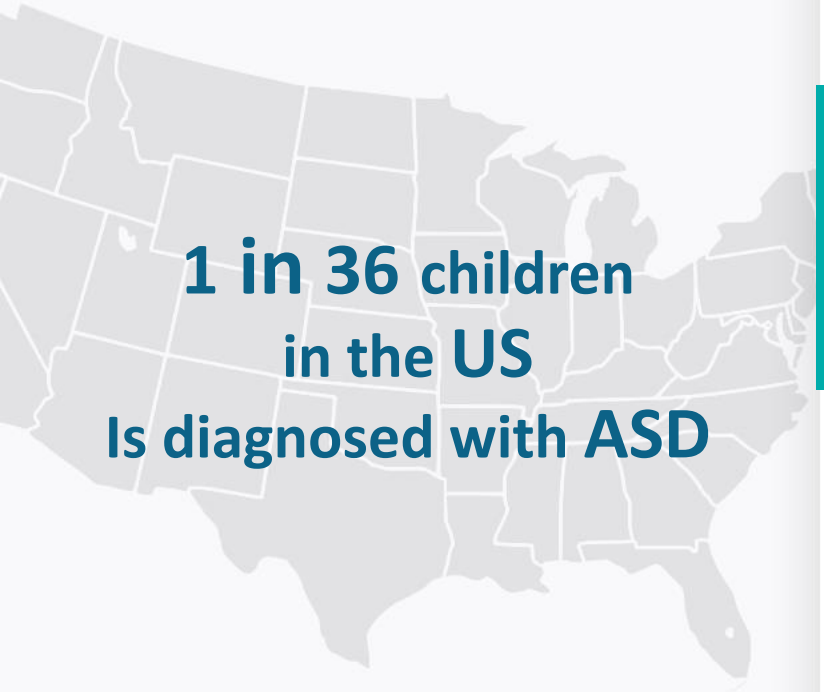
PAX-101, PAX-102, Emodin

Potential Game-Changing Treatments for Autism

Autism Spectrum:
A Significant Clinical Opportunity



1 in 36 children in the US Is diagnosed with ASD

No FDA-approved treatments for Autism core symptoms:

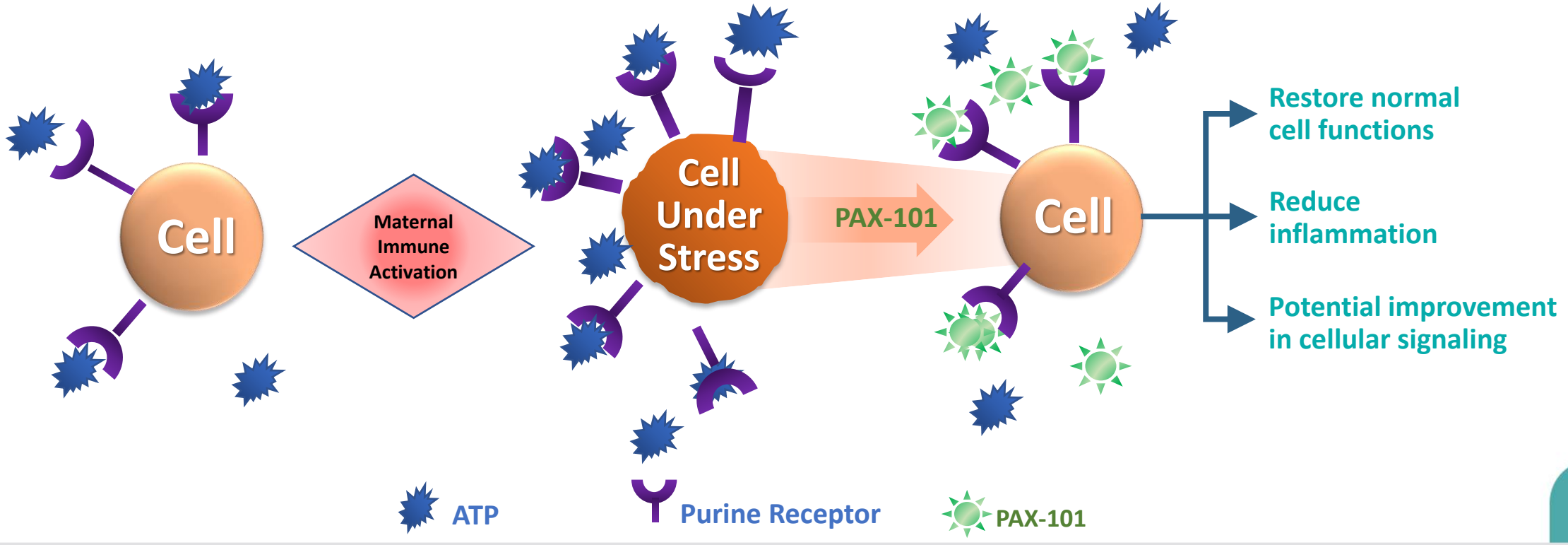
- Deficits in Social Communication
- Restricted and Repetitive Patterns of Behavior
- Significant Impairment in Functioning

- **Global Autism treatment market reached $3.3B in 2018 and is expected to exceed $4.6 Billion in 2026 (CAGR 4.3%)[1]**
  - Irritability, a non-core symptom associated with ASD, is treated with available anti-psychotic drugs but tolerability and side effects can be treatment limiting

1. Fortune Business Insights, Autism Spectrum Disorder Therapeutics Market Research Report, August 2019

A New Treatment Pathway Proposed for Autism

PAX-101 may improve symptoms of ASD by blocking the action of ATP on purine receptors that are over-expressed during immune system activation



An Expanding Pipeline of Anti-Purinergic Compounds

Compound	Indication	Pre-Clinical	Phase 1	Phase 2	Phase 3	NDA
PAX-101	Stage 1 Trypanosoma Brucei Rhodesiense Human African Trypanosomiasis					
PAX-101	Autism Spectrum Disorder					
Emodin	Autism Spectrum Disorder					
PAX-102	Autism Spectrum Disorder					

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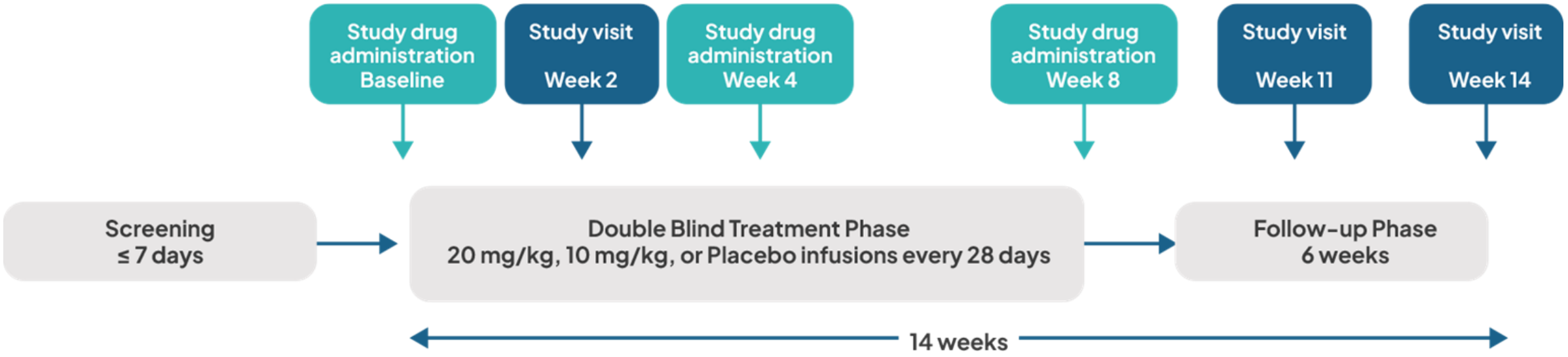
Forging a New Path in ASD Treatment:

PAX-101 Phase 2 Trial in ASD

PAX-101 in ASD Phase 2 Clinical Trial Design

Study Diagram

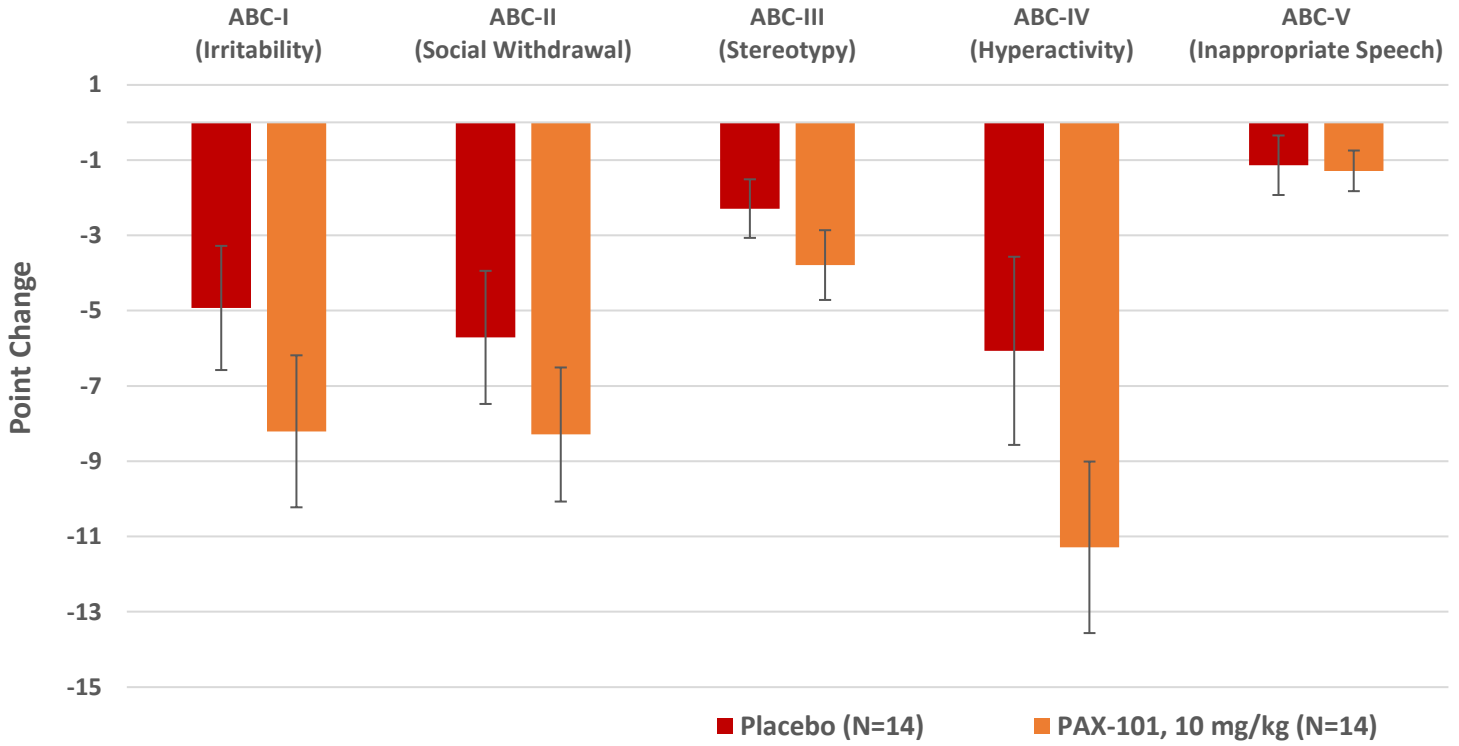


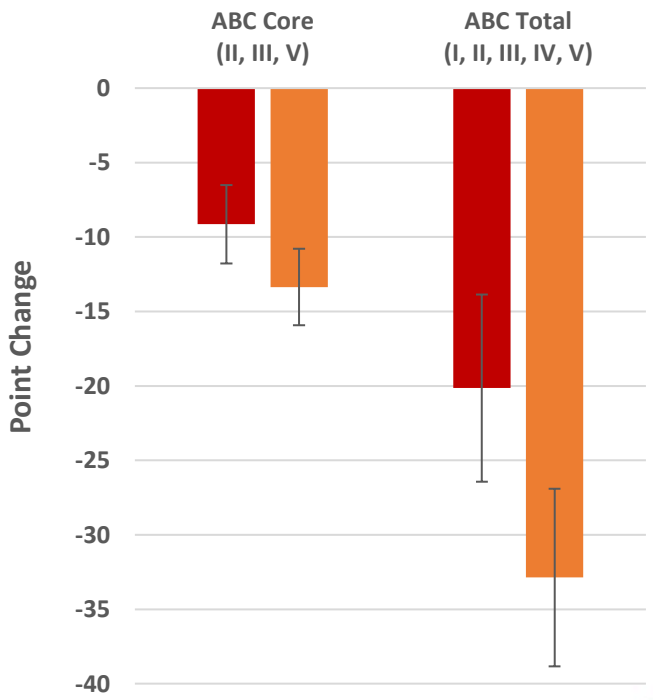
6 sites in Africa – 44 boys completed study – mean age 8.4 years

Primary Endpoint: Aberrant Behavior Checklist (ABC)

Positive Individual ABC Subscale Changes

Change from Baseline through Week 14

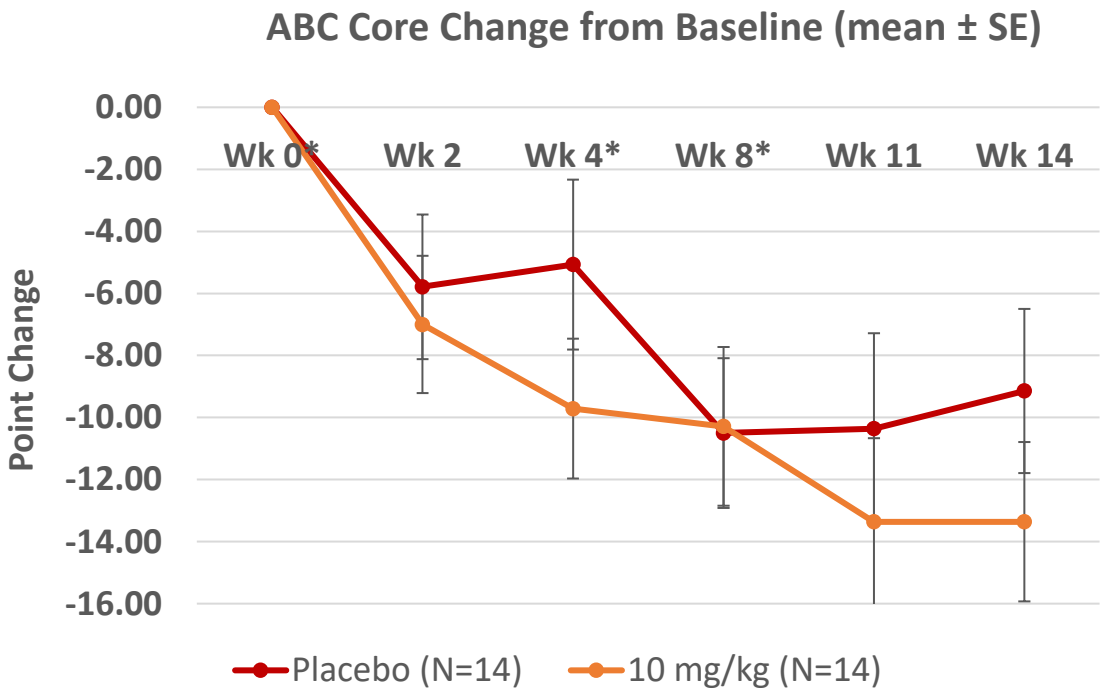


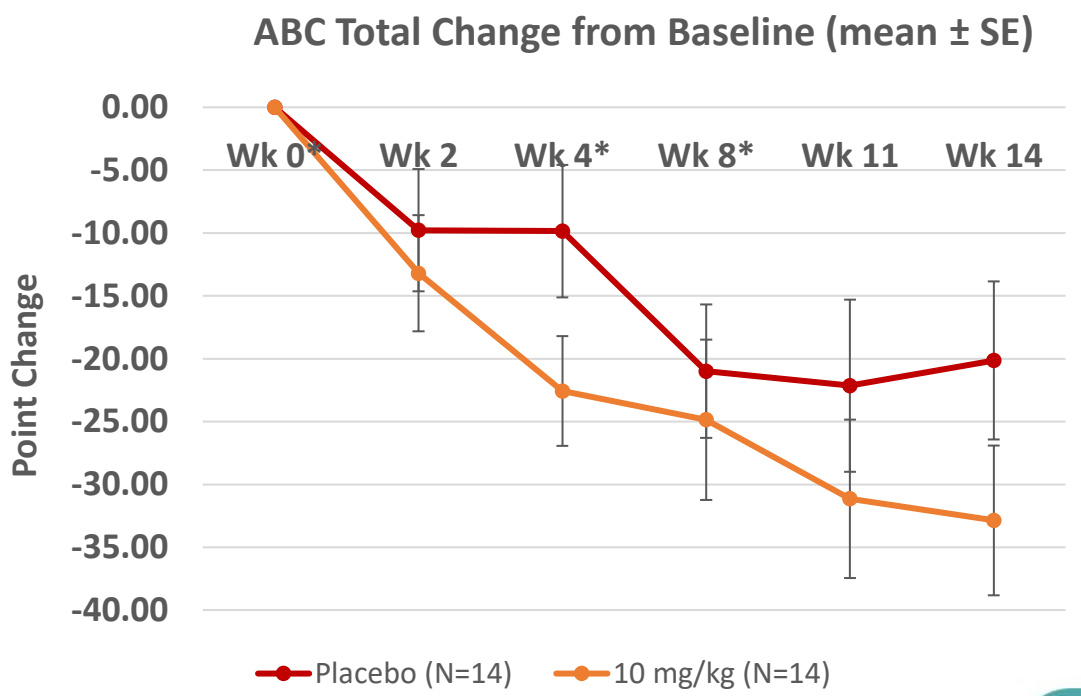


Primary Endpoint: Aberrant Behavior Checklist (ABC)

Core Symptoms and Total Symptoms – Positive Change from Baseline over time

PAX-101 Low Dose (10mg/kg) Outperforms Placebo in Core and Total Symptoms Measures



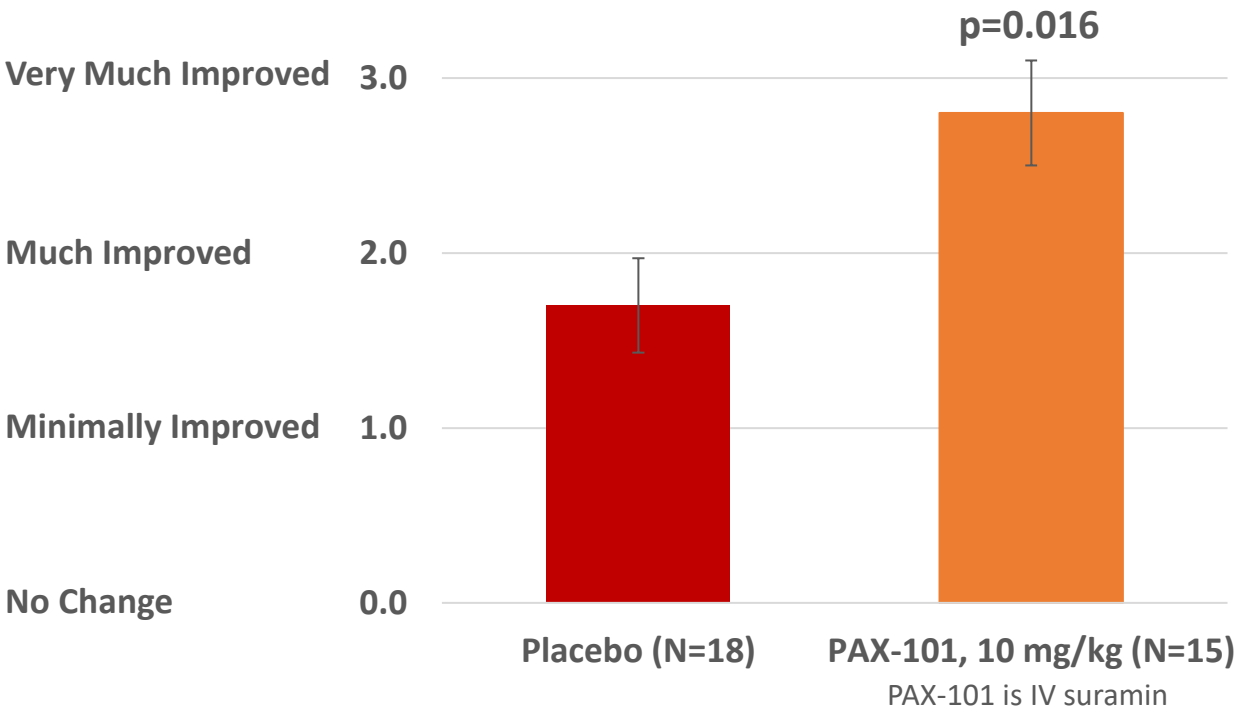


* Drug administration visit

Secondary Endpoint: Clinical Global Impression of Improvement in Overall Severity of Symptoms – Positive Change from Baseline

CGI-I Overall Severity Score, Scaled

Change from Baseline to Week 14 (ITT population, mean ± SE)



PAX-101 is IV suramin

Results	Chg from BL	P Value	Adj P Value
10 mg/kg	-2.8 ± 0.30	0.008	0.016
Placebo	-1.7 ± 0.27		

PAX-101: Phase 2 Trial Summary Results

- Both dose regimens showed improvement trends from baseline on ASD core symptoms
- 10 mg/kg dose group showed a statistically significant and clinically meaningful change on the CGI-I
- Confirmation of these results needed in a larger, statistically powered study
- Preparing for next clinical trial of PAX-101 in ASD

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PAX-101 for HAT
NDA Submission Plans

Filing NDA for HAT in Parallel with ASD Development Work

Priority Review Voucher Program for Neglected Tropical Diseases

- IV suramin is the standard of care in the treatment of potentially lethal infections caused by Stage 1 T. Brucei Rhodesiense Human African Trypanosomiasis (HAT), aka African Sleeping Sickness, for over 100 years
- Currently only approved in Africa
  - PaxMedica has the exclusive license to suramin-treated patient data from key endemic hospitals
- PAX-101 received FDA Orphan Drug Designation for this neglected tropical disease in Nov. 2020
  - If approved would mean market exclusivity up to seven (7) years
- NDA Sponsors under this program may qualify for FDA benefits, including a Priority Review Voucher. PRV Vouchers can potentially be sold to a third party upon receipt following NDA approval*
- PaxMedica intends to file an NDA for the use of PAX-101 in HAT infections in 2024
  - HAT submission will be a 505(b)(2) – NDA
  - If approved, will be the first approval of suramin in the US market

* For full analysis see GAO Report GAO-20-251, as of January 31, 2020

PAX-HAT-301: Registrational Phase 3 Trial in Rare Tropical Disease

Top line Results	• Suramin-treated cohort 114 (94%) survived and completed treatment • Natural History cohort 6 (14%) recorded as cured, improved or discharged, 3 (7%) died, 10 (24%) experienced clinical worsening, 17 (40%) achieved moribund status (near death or in terminal decline) • The two-sided p-value for the Fisher's exact test was <0.001
Study Design	• Retrospective clinical study to determine efficacy and safety of the use of PAX-101 (suramin) to treat Stage 1 Trypanosoma Brucei Rhodesiense Human African Trypanosomiasis (TBR HAT) • 121 in the suramin-treated cohort, 42 in the natural history cohort
Objectives	• Did the suramin treated cohort have better outcomes than those in the Natural History cohort? • Any recorded treatment side effects?
Next Steps	• PaxMedica expects Q4 meeting with FDA to discuss updated path to NDA.

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Recent Accomplishments
Goals and Financials

Recent Accomplishments

- Announced Positive Top Line Results from the PAX-101 (intravenous suramin) Phase 3 African Sleeping Sickness Study, PAX-HAT-301
- Granted Exclusive Pharmacy Distribution Rights for PAX-101 to Vox Nova, LLC
  - First purpose-built limited distribution specialty pharmacy benefit manager that is focused on ASD
  - Establishing initial infrastructure necessary to distribute PAX-101 safely and effectively
- CMC Supply Chain – API and Drug Product cGMP Process on track to be completed by year end 2023
- Entered into Research Collaboration Agreement with PoloMar Health and The BRAIN Foundation for Phase II Study in ASD
  - Phase II proof-of-concept trial using a proprietary, highly bioavailable form of emodin in patients with ASD is expected to begin in 2H 2023
  - Positive results were demonstrated in several in vivo animal studies in a mouse model of autism conducted by PaxMedica in 2019

2023-2024 Goals

- Reduce cost of capital and dilution as we approach NDA and potential PRV
- Complete all necessary pre-clinical, non-clinical and clinical studies to support NDA submission for HAT indication
  - ✓ Announced Positive Top Line Results from the PAX-101 (intravenous suramin) Phase 3 African Sleeping Sickness Study
- Fund company through 2025
- Planning for FDA meeting in Q4 2023 for HAT
- Complete manufacturing validation of PAX-101 in preparation for NDA submission in 2024

Ample Capital Available for Near-term Needs

- As of June 30, 2023
  - $3.1 million in cash on balance sheet
  - Remaining access of approximately $15.2 million from committed equity investment agreement for up to $20 million with Lincoln Park Capital
  - 15.4 million shares of Common Stock outstanding
- Potential for award of U.S. Rare Tropical Disease Priority Review Voucher (PRV)

Key Takeaways

- PaxMedica is the only public company focused on developing treatments for the core symptoms of ASD
- Multiple catalysts expected through 2024
  - Planning a multi-centered clinical trial of PAX-101 in ASD
  - Preparing NDA filing for approval of PAX-101 for the treatment of Human African Trypanosomiasis (HAT), a rare and fatal tropical disease
    - Expect to file in 2024
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THANK YOU!

WWW.PAXMEDICA.COM

For More Information Contact:
Buzz Woods
PaxMedica Corporate Communications
bwoods@paxmedica.com

Stephanie Prince
PCG Advisory
sprince@pcgadvisory.com
(646) 863-6341