

Identifying value drivers for a novel treatment targeting core symptoms of Autism Spectrum Disorder

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Introduction

- Autism spectrum disorder (ASD) is a highly heterogenous neurodevelopmental condition that affects communication skills, social interaction, learning skills, and behavior
- There are currently no pharmacological treatments indicated for the core symptoms of ASD, which are defined as social communication deficits and repetitive restrictive behavior by DSM-V
- Treatment is often based on patients’ access to behavioral therapy and the management of associated symptoms through off-label use of antipsychotics, SSRI's, and other medicines, which often come with significant adverse events
- The medical burden for ASD is high, more than 70% of individuals with ASD have at least one current associated condition e.g., anxiety, depression, and ADHD
- This research explores payer and physician perceptions of the unmet need in ASD and what stakeholders view as the most important value drivers for a potential novel treatment targeting core symptoms of ASD

Methods

- A total of 35 Payers and 15 physicians were interviewed across US, Germany, France, Canada, and Japan to assess the remaining unmet need in ASD, evaluate current treatment options, and identify the value drivers for a potential novel treatment for the core symptoms of ASD
- Physician and Payer DYADS interviews were undertaken in each country (n=7 US, n=2 ex-US) followed by in-depth individual Payer TDI’s (n=8 US, n=3 ex-US)

Table 1: Stakeholders recruited by country

	Payer	KOL
US	n=15 (National & Regional MCOs, National PBMs, Regional IDNs)	n=7 (Neurologists, Psychiatrists, Psychologists, Developmental Pediatrician)
Canada	n=5 (Provincial & Private payers, advisors and ex-payers)	n=2 (Psychiatrist, Neurologist)
France	n=5 (Ex-members of CT, Ex-member of CEPS)	n=2 (Psychiatrist, Neurologist)
Germany	n=5 ((Ex)-member of the G-BA, IQWiG advisor, Former KK)	n=2 (Psychiatrist, Neurologist)
Japan	n=5 (Ex-member of MHLW, ex-member of Central Social Medical Insurance Council)	n=2 (Psychiatrist, Neurologist)
Total	n=35	n=15

Results

Figure 1: Average stakeholder rating of unmet needs for ASD

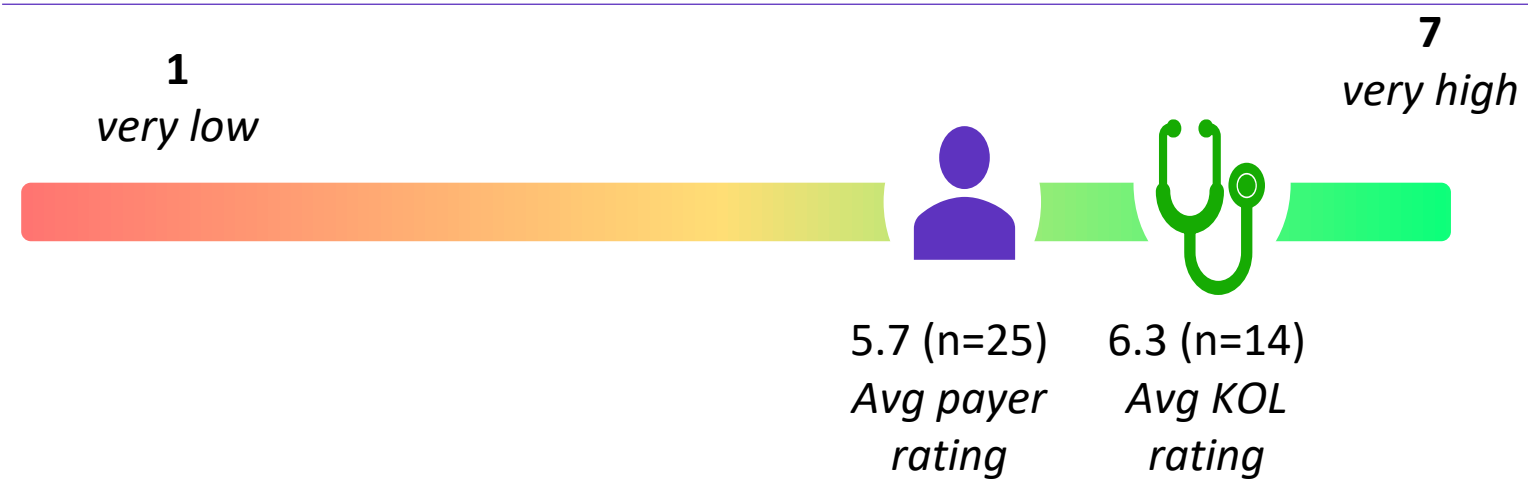


Figure 2: Top unmet needs identified for ASD

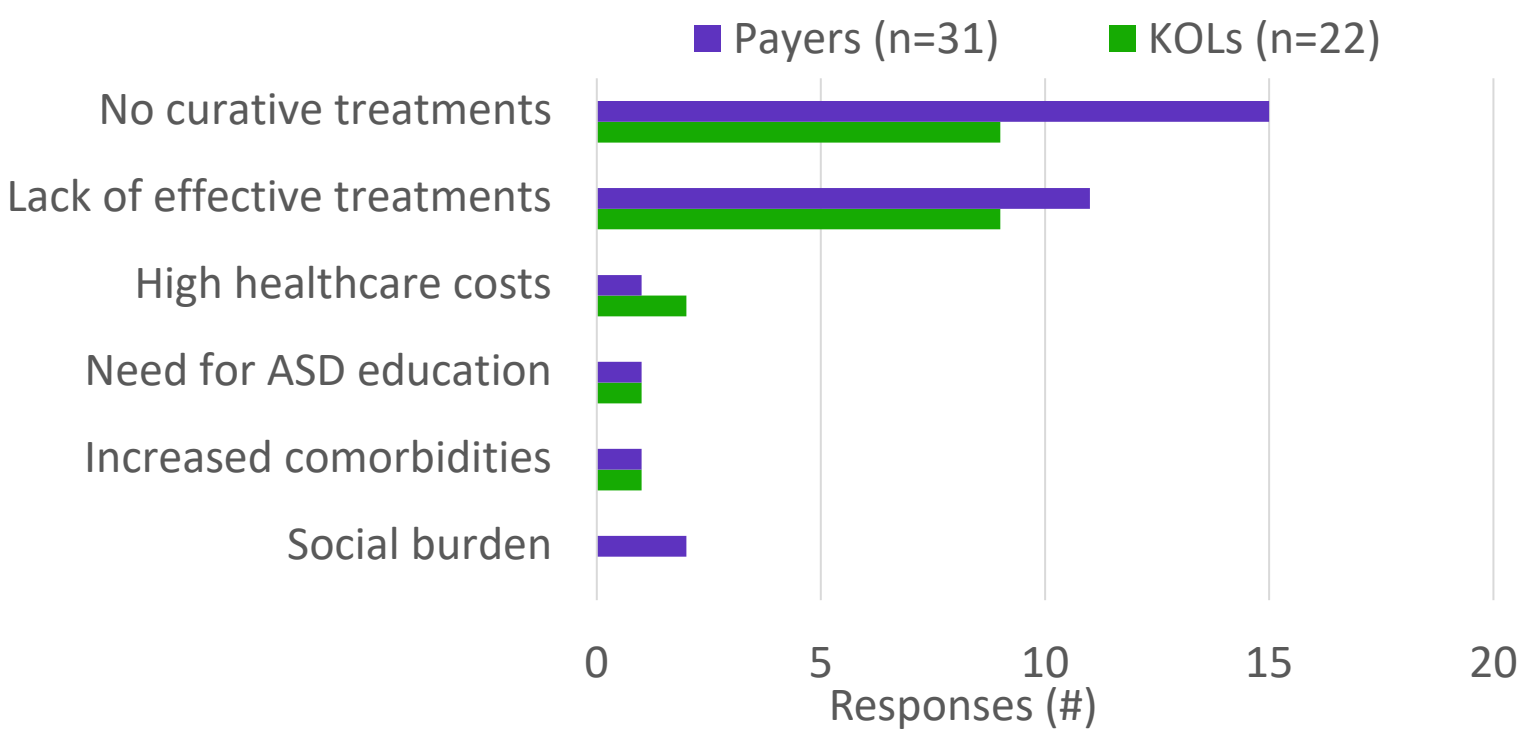
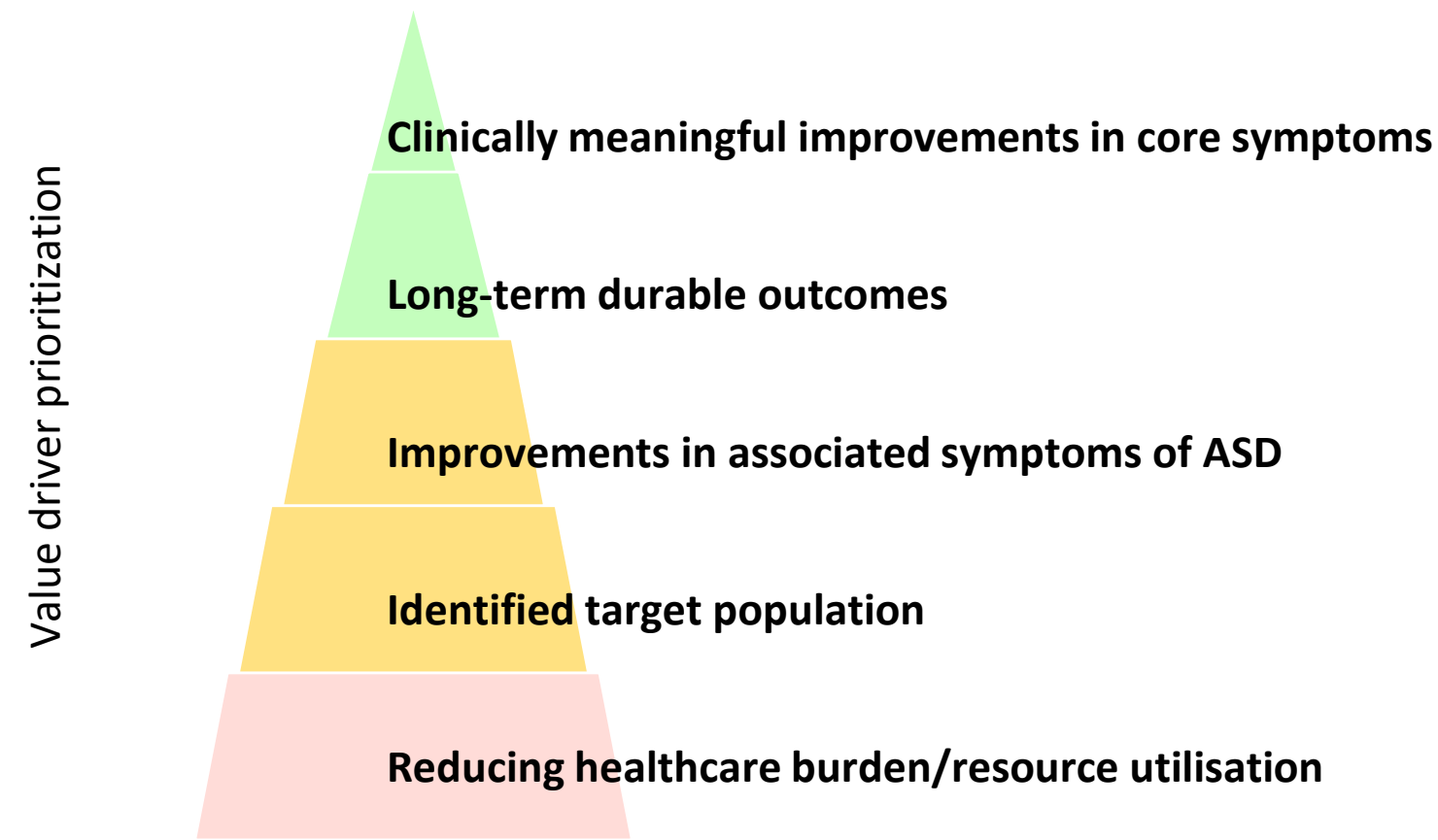


Figure 3: Overall Value Driver prioritization across all stakeholders



Key Findings:

- Improvements in social communication, and ability to engage at work/school and a decrease in repetitive behaviors are desirable outcomes for a novel therapy, as stakeholders perceived these to present the highest burden to patients
- Additionally, stakeholders across all markets agreed there is a high unmet need in ASD driven primarily by the lack of treatment options targeting core symptoms; improvements in core symptoms was therefore weighted the most important value driver (n=26 Payers, n=15 KOLs)
- Long-term durable outcomes was identified as another value driver across all markets, both to demonstrate long-term efficacy but also long-term safety and tolerability, as treatments are expected to be life-long and the use of off-label anti-psychotics is often accompanied by significant side effects, such as weight gain and metabolic syndrome
- Most stakeholders (n=11 Payers, n=7 KOLs) recommended that a novel therapy for ASD should have a pivotal trial with a minimum 6-month duration and a 1-year open label follow-up to optimize HTA and reimbursement opportunities

- Furthermore, improvement in associated symptoms, such as anxiety, depression, sleep disorders and gastrointestinal issues was a highly important value driver for Japan (n=4 payers), and a moderately important value driver for the US, Canada, France, and Germany (n=15 payers)
- KOLs and Payers across all markets wanted to understand the effectiveness of a treatment vs. the current SoC within clearly defined patient populations, categorized by age, symptom-severity, and associated symptoms (e.g., anxiety/depression) given the disease heterogeneity
- Reduction in healthcare burden / resource utilization as a value driver varied across regions, and payers in the US, France, Japan, and Canada perceived this as moderately important; however, German payers are not as likely to evaluate economic impact given this is not included in the G-BA’s assessment; additionally, payers justified a lower prioritization rating for this metric as behavioral therapy is the current standard of care across several markets and is funded separately to pharmacotherapies
- High costs are driven by ineffective treatments for associated symptoms, high costs for non-pharmacological treatments, such as behavioral therapy and educational and social support, management of comorbidities and polypharmacy, etc.

Conclusion

- There is a high disease burden for people living with ASD given the lack of novel treatments targeting core symptoms
- With the growing prevalence of ASD across the globe, there is a remaining unmet need for effective therapies
- For a manufacturer looking to launch a novel ASD pharmacotherapy, the most important factor to consider is long term improvement in core symptoms without compromising on safety or QoL outcomes compared to the current SoC (behavior therapy + pharmacotherapy)

References

Clarivate Consulting Primary Research
Abbreviations: Autism Spectrum Disorder (ASD), Attention Deficit Hyperactivity Disorder (ADHD), Key Opinion Leader (KOL), Dual interview with Payer and KOL (DYAD), telephone-directed interview (TDI)