EXPERT REPORT AUTISM SPECTRUM DISORDERS – MARKET OPPORTUNITYAND PHARMACOLOGICAL STRATEGIES

Main period covered 2007-2012; 130 pages; 300 references		
		Part 1: MARKET OPPORTUNITIES Prevalence, Patient Numbers, Clinical Need Part 2: PHARMACEUTICAL STRATEGIES Present and Future
		An authoritative review of the prevalence, symptomatology and clinical need around Autism Spectrum Disorders, the positioning of current pharmaceutical interventions and the new scientific hypotheses around the aetiology of autism and novel approaches for drug discovery and clinical development.
		Reasons to purchase
		• Benefit from the <i>exceptional insight</i> and experience of a scientist embedded in the field.
		• Gain immediate insight into <i>market opportunity</i> by understanding prevalence
		and current interventions.
		• Understand the <i>unmet clinical need</i> for core autistic symptomatology and
		commonly associated mental health problems.
		• Use the summary of the neurobiology of neurodevelopment disorders to
		understand the implications for <i>drug discovery and clinical development</i> .
		• Understand the hypotheses behind the <i>validation of novel pharmaceutical</i>
		therapeutic targets.Discover how pharmaceutical companies are already exploiting
		opportunities.

NeuroKnowledge Limited

EXPERT REPORT AUTISM SPECTRUM DISORDERS

1. MARKET OPPORTUNITY

PREVALENCE, PATIENT NUMBERS, CLINICAL NEED

2. PHARMACEUTICAL STRATEGIES

PRESENT AND FUTURE

Reviews:

- The status of interventions used in autism spectrum disorders, how pharmacological medicines sit within this framework and the potential benefits that pharmacologics can bring.
- The level of empirical evidence around existing pharmacological intervention.
- Clinical unmet need arising from the core deficits of autism spectrum disorders and other symptomatology and behaviours.
- How advances in understanding the aetiology of autism, together with profound discoveries in the neurobiology of related developmental disorders, have generated testable hypotheses and tractable pharmacological targets.
- Insight into drug discovery and the development of new medicines.
- Expectations of increasing demand and a growth market, and the importance of lessening the impact of autism spectrum disorders within the wider healthcare, social and economic contexts.

TABLE OF CONTENTS

Part 1: MARKET OPPORTUNITY

PREVALENCE, PATIENT NUMBERS, CLINICAL NEED

Section 1 EXECUTIVE SUMMARY

Section 2 GOALS FOR INTERVENTION IN AUTISM SPECTRUM DISORDERS

Section 3 ABOUT AUTISM SPECTRUM DISORDERS

- 3.1 Definitions
- 3.2 Descriptions
- 3.3 Special abilities
- 3.4 Diagnosis
- 3.5 Economic impact

Section 4 POTENTIAL PATIENT NUMBERS

- 4.1 Prevalence
 - 4.1.1 Increase in number of reported cases
 - 4.1.2 Worldwide prevalence
 - 4.1.3 Number of affected individuals the potential patient pool
- 4.2 Will the number of diagnosed ASD cases increase?
- 4.3 Are autism and ASDs considered 'rare' disorders?
- 4.4 Comparison of ASD prevalence with other neurodevelopmental disorders and insight into market opportunity
- 4.5 Mental health conditions and other symptomatology associated with ASDs
- 4.6 Extent of association of ASDs with other mental health problems

Section 5 INTERVENTIONS

- 5.1 Consumer satisfaction
- **5.2** Empircal evidence
- 5.3 Medicines used to manage ASDs
 - 5.3.1 Core deficits
 - 5.3.2 Irritability and the use of atypical neuroleptics
 - 5.3.3 Medicines used to manage associated mental health conditions

Section 6 UNMET CLINICAL NEED

- 6.1 Improving diagnosis
- 6.2 Addressing core deficits
- 6.3 Removing barriers by limiting problematic behaviours
- 6.4 Helping improve and reduce the stress on family life
- 6.5 Life span relevant benefits children, adolescents and adults

Part 2: PHARMACOLOGICAL STRATEGIES

PRESENT AND FUTURE OPPORTUNITIES

Section 7 ADVANCES IN AETIOLOGY

7.1 The brain

- 7.1.1 Brain structure and function
- 7.1.2 Under the microscope
- 7.1.3 Summary the brain

7.2 Genetic and non-genetic causes

- 7.2.1 Genetic variation and susceptibility to ASD
- 7.2.2 Syndromic autism
- 7.2.3 Rare or common genetic variation?
- 7.2.4 Genetic base diagnostics
- 7.2.5 Functional impact of genetic variation autism as a synapsopathy
- 7.3 Summary: ASD as disordered brain organisation and connectivity

Section 8 PHARMACOLOGICAL INTERVENTIONS – LOOKING BACK

- 8.1 Diversity of clinical pharmacology research
- 8.2 Atypical neuroleptics
- 8.3 Other commonly used medicines
- 8.4 Status of empirical evidence in clinical pharmacology

Section 9 LOOKING TO FUTURE OPPORTUNITY AND NEW MEDICINES

- 9.1 Potential line extensions and repurposing
- 9.2 Opportunities for repurposing ASD as an additional therapeutic indication

Section 10 NOVEL PHARMACOLOGICAL TARGETS REVEALED

10.1 Trailblazer studies and commercial development activity in developmental disorders

- 10.1.1 Fragile X and the glutamatergic (mGluR5) hypothesis
 - Commercial development activity
- 10.1.2 Tuberous Sclerosis Complex and mTOR signalling
 - Commercial development activity
- 10.1.3 Rett's syndrome and MeCP2
 - Commercial development activity

10.2 Evidence and commercial development activity for other tractable pharmacological targets in ASD

- 10.2.1 GABA
- 10.2.2 Glutamate
- 10.2.3 Oxytocin and vasopressin
- 10.2.4 Serotonin
- 10.2.5 Melatonin sleep, circadian rhythms and clock genes
- 10.2.6 Biomarkers

Section 11 MARKET FORECASTS

Section 12 ACTIVE COMPANIES IN ASD DIAGNOSTICS AND THERAPEUTICS

- 12.1 Molecular Diagnostics Companies
- 12.2 Pharmaceutical Companies

Part 2: continued

Section 13 PATENT LANDSCAPE

CONCLUSIONS

BIBLIOGRAPHY

EXHIBITS

- 1. Neurocognitive theories of autism
- 2. Brief descriptions of Pervasive Developmental Disorders or Autism Spectrum Disorders
- 3. Adult outcomes in individuals who received a diagnosis of typical autism in childhood
- 4. Number of children and young adults served by the Individuals with Disabilities Education Act (IDEA) in the USA
- 5. Example of a new calculation of the number of people affected by ASD
- 6. The pool of typical autism cases and description of need
- 7. Comparison of the potential pharmaceutical market for ASD with the known markets for ADHD and schizophrenia
- 8. Attention and mood issues in children with ASDs
- 9. Examples of the range of interventions used in the UK for the management of autism spectrum disorders
- 10. Researching interventions in ASD and priorities for research
- 11. Categories of medication used by children with ASD
- 12. Summary of clinical pharmacology studies in neurodevelopmental disorders
- 13. Statement from President Obama on World Autism Awareness Day 2009
- 14. World Autism Awareness Day UN Resolution

APPENDICES

- 1. Summary of clinical pharmacology studies in neurodevelopmental disorders
- 2. Short description of some commonly used rating scales and diagnostic and research instruments
- 3. Summary of patent activity in autism spectrum disorders