

medgenics



UBS Healthcare Conference

May 25, 2016

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Forward Looking Statement

This presentation includes certain estimates and other forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, including statements with respect to anticipated operating and financial performance, clinical results, potential partnerships, licensing opportunities and other statements of expectation. Words such as “expects,” “anticipates,” “intends,” “plans,” “believes,” “assumes,” “seeks,” “estimates,” “should” and variations of these words and similar expressions, are intended to identify these forward-looking statements. While we believe these statements are accurate, forward-looking statements are inherently uncertain and we cannot assure you that these expectations will occur and our actual results may be significantly different. These statements by the Company and its management are based on estimates, projections, beliefs and assumptions of management and are not guarantees of future performance. Important factors that could cause actual results to differ from those in the forward-looking statements include the factors described in the Company’s filings with the U.S. Securities and Exchange Commission. The Company disclaims any obligation to update or revise any forward-looking statement based on the occurrence of future events, the receipt of new information, or otherwise.

Executive Summary

- Enthusiastic initial response to our genomic medicine approach in ADHD from physicians and patients
- Rapid enrollment into phenotype/genotype study provides platform for rapid execution of Phase 2/3 trial in mGluR+ ADHD adolescent trial
- Prevalence rates for mGluR+ mutation in ADHD patients age 6-17 confirmed at approximately **25%**
- Based upon mGluR mutation prevalence, conservative market opportunity for NFC-1 in the US is **\$2-3B**

Medgenics/CHOP Collaboration

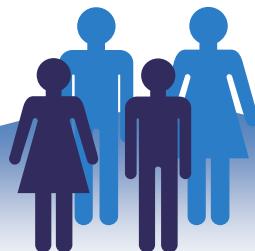
- Medgenics has an **exclusive option** to the **genomic discoveries** made by the Center for Applied Genomics (CAG) in **Rare and Orphan diseases**
- CAG is prolific in discovering novel **genetic causes of disease**...however...translating **discoveries** into **therapies** has been a challenge
- Medgenics' role is to guide the **translational process** that matches **genetic discoveries** with **potential therapies**, and accelerates them into the clinic

*Our collaboration with CAG gives us **proprietary insight** into the underlying genetic causes of disease which enables discovery of **novel targets in genetically distinct populations** with high unmet medical need.*

CAG/CHOP Capabilities

CAG's pediatric biobank contains a high percentage of rare genetic variants

- ~1.2M patient visits/year
- 10% of all R/O disease patients in N. America are treated at CHOP



- Population is unique in that it represents the most severe forms of common diseases
- Global reach in many therapy areas

Highly scalable infrastructure to support translational research



Biobank (BB)

- Fully automated robotic biorepository

Datasets (Genomics EMR)

- Over 60K pediatric and 150K related adult patients GWAS genotyped with associated longitudinal EMR since 2006

Data Analytics

- End to end internal Next-Gen sequencing capabilities
- Integrated bioinformatics
- Rapid identification of novel genetic biomarkers

Consented Patients

- 85% of the BB patients are consented for longitudinal follow up and are eligible for call back for future studies

In the last 8 years CAG has had over 400 peer reviewed publications focused on novel genetic discoveries

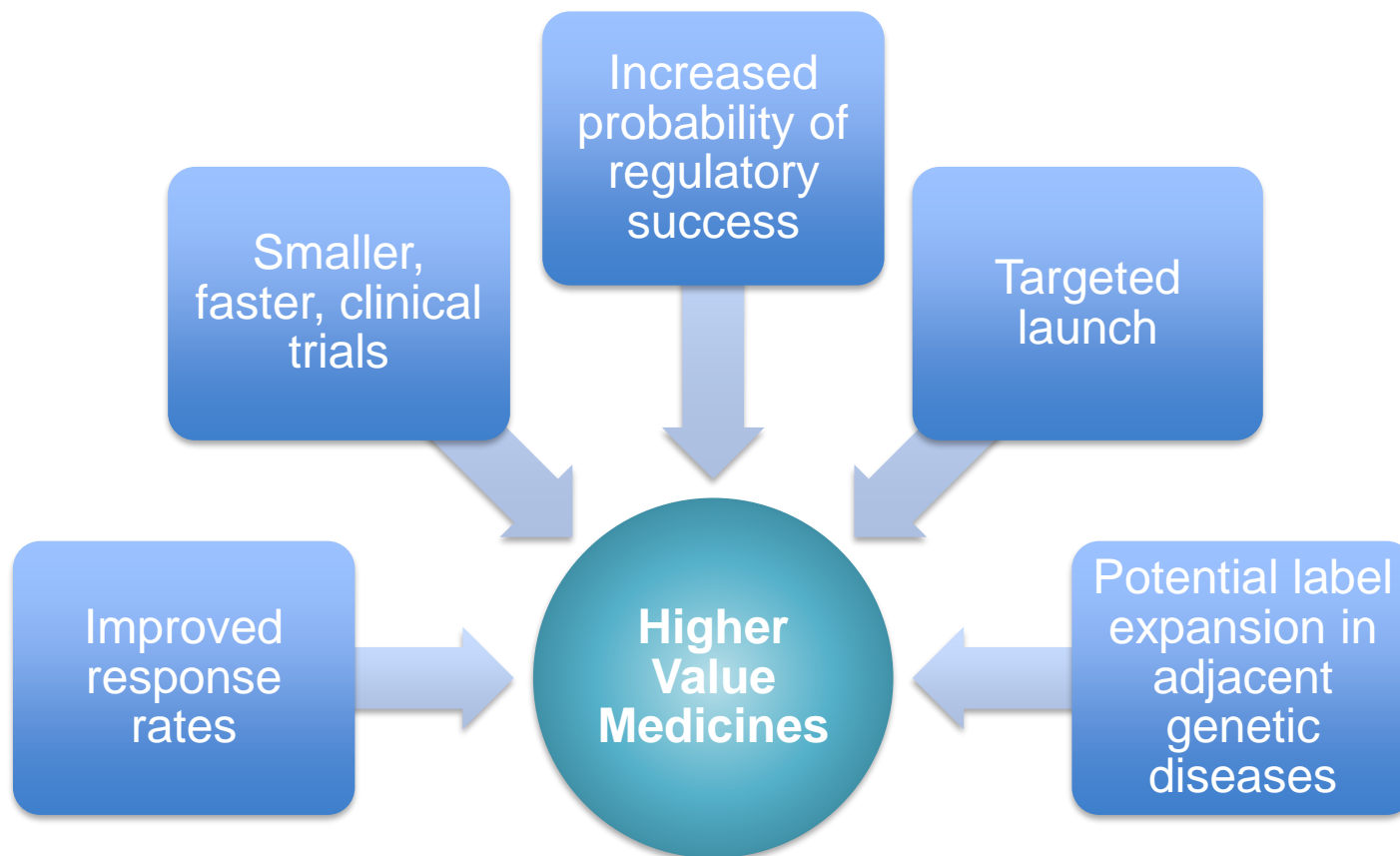
MDGN/CAG Drug Development Strategy



- Novel targets are validated, and then a search begins for an existing molecule with the correct MOA that can be repurposed
 - An ideal program will be clinical stage with an excellent safety profile and a suboptimal efficacy signal based on studying heterogeneous patient populations
- Once a drug is identified and in-licensed, a rapid proof of concept trial is conducted
 - Genetically stratified and consented patients are called back

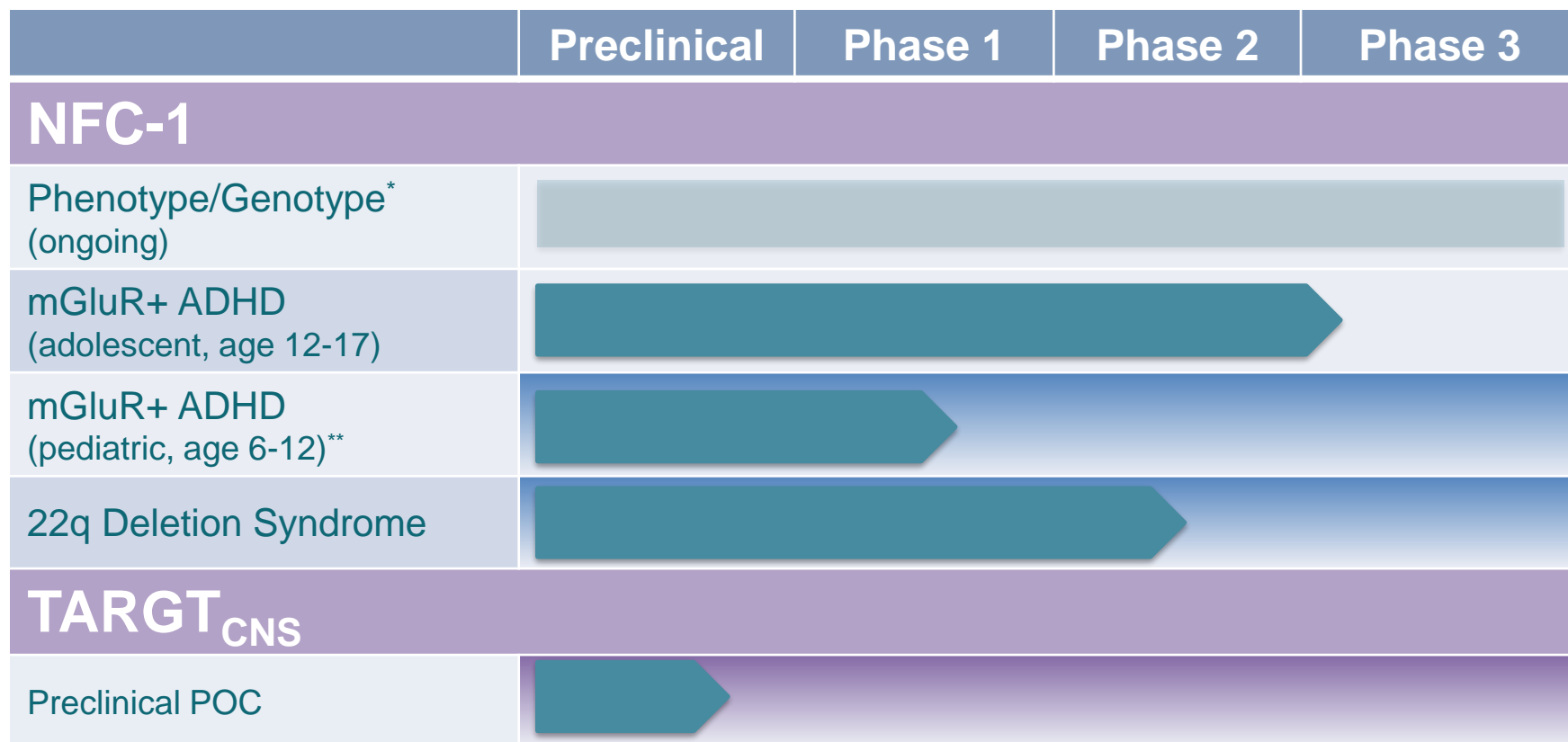
Benefits of Genomic Guided Drug Development

Genomic biomarkers improve overall program outcomes



NFC-1 Development Program Update

Development Pipeline



* Non-interventional study to screen and identify ADHD patients for phase 2/3 program

** Initiate Phase 3, H1 2017

NFC-1 ADHD Program

**ADHD Phenotype/Genotype
Pediatrics, Adolescents
N=1,000+**

Design:

- Non-interventional
- Multi-center (25)
- Genotype 1,000+ ADHD subjects, age 6-17, identifying patients who are mGluR+

Objectives:

- Confirm prevalence of mGluR+
- Expedite enrollment in Phase 2/3 interventional trials

Timing: Ongoing

**Phase 2/3
mGluR+ ADHD, Adolescents
N=90, Ages 12-17**

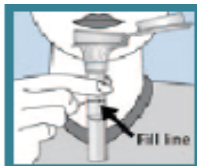
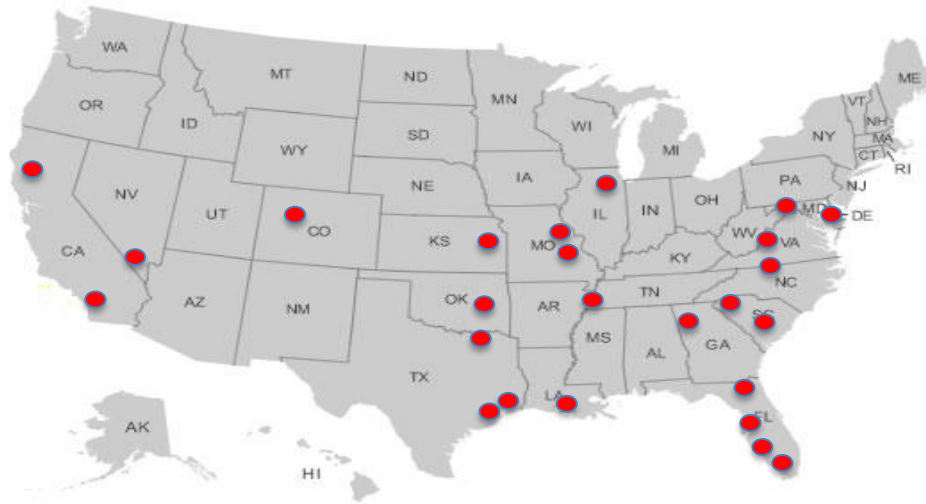
**Phase 3
mGluR+ ADHD, Pediatrics
N= TBD, Ages 6-12**

**Phase 3
mGluR+/- ADHD, Ages 6-17
N=TBD**

ADHD Phenotype/Genotype Study

25 ADHD Investigational Sites Across the U.S.

- 1,000+ ADHD patients ages 6-17 years



Saliva Collection

- Study site obtains saliva sample and sends to CAG for DNA extraction



Consented Patients

- 95% of the study subjects have agreed to be contacted for future studies



CHOP/CAG Biobank

- DNA extraction
- Genetic Sequencing
- BioBanking of DNA



Medgenics Database

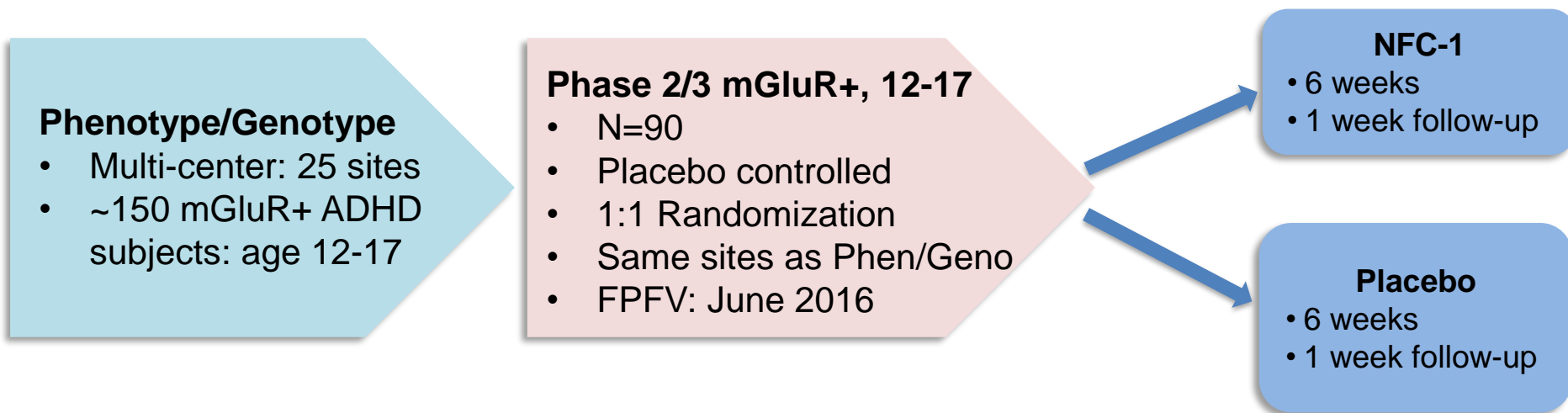
- Evaluation of genotype and phenotype data
- Identification of subjects for future trials

Phenotype/Genotype Study – Interim Results

- Rapid enrollment
 - More than **700 subjects genotyped** in the first **10 weeks** at more than **20 US sites**
 - Enrollment to complete in June 2016
- Confirmation of mGluR mutation prevalence
 - Approximately **25%** of ADHD patients age 6-17 are **mGluR+**
 - Validation of previous CAG findings
- **95%** of subjects **consented** to contact regarding future interventional trials
- Enthusiastic response from clinicians and patient community

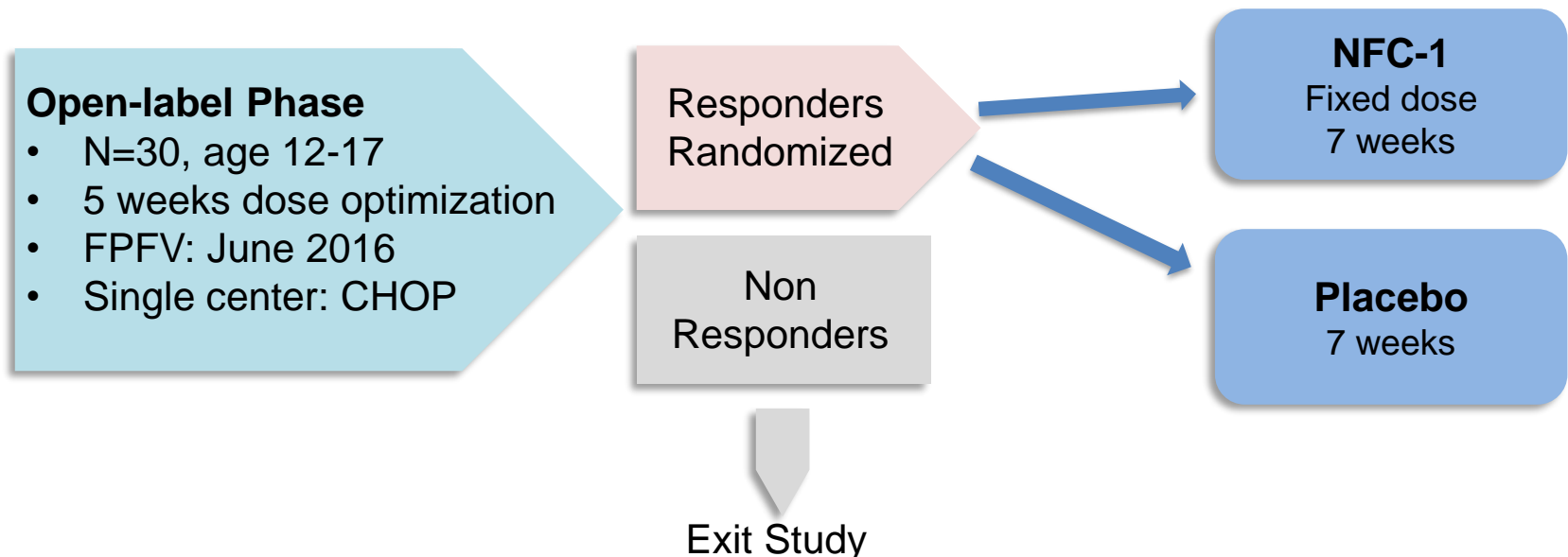
Phase 2/3 Study in Adolescents with mGluR+ ADHD

- Objective:
 - Optimize dose in adolescents, confirm Phase 1B results
 - Endpoints: ADHD-RS, CGI-I
- FPFV June 2016
- H2 2016: Topline Data



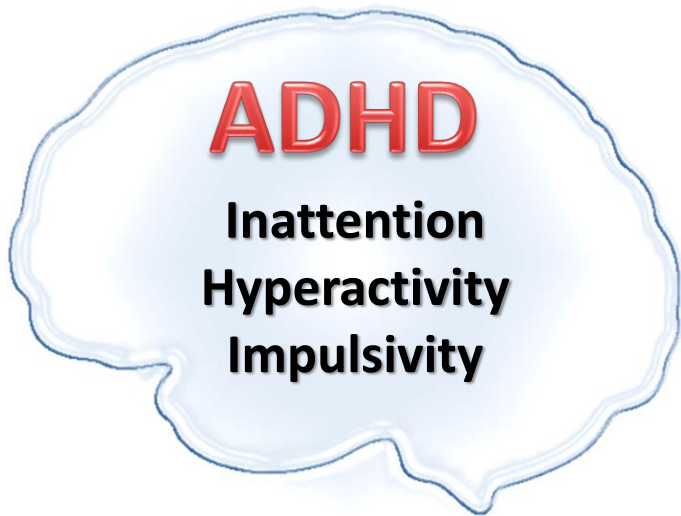
Phase 1/2 Study in 22q Deletion Syndrome

- Objective:
 - Explore symptoms from three neuropsychiatric disorders: ADHD, Anxiety, and Autism Spectrum Disorders (ASD)
 - Endpoints: CGI-I, ADHD-RS, PARS, CARS-2
- FPFV June 2016 (pending final CHOP approval)
- H2 2016: Responder rate data from open-label phase



mGluR+ ADHD Market Opportunity

ADHD: Background and Rationale for Genomic Medicine Approach



- Most prevalent neurodevelopmental disorder in children
- Highly heritable* (70%); Heterogeneous, suggesting multiple causes
- Early studies, focused on individual genes, failed to identify an underlying cause
- Based upon network biology and genotyping, CAG described genetic mutations identifying a novel subset of inherited ADHD**
- mGluR network disruption mimics Mendelian inheritance and accounts for ~25% of ADHD cases in children

* Farone SV, Perlis RH, Doyle AE, et al. Molecular genetics of attention-deficit/hyperactivity disorder. *Biol Psychiatry* 2005; 57: 1313-1323

** *Nature Genetics* 44, 78–84 (2012).

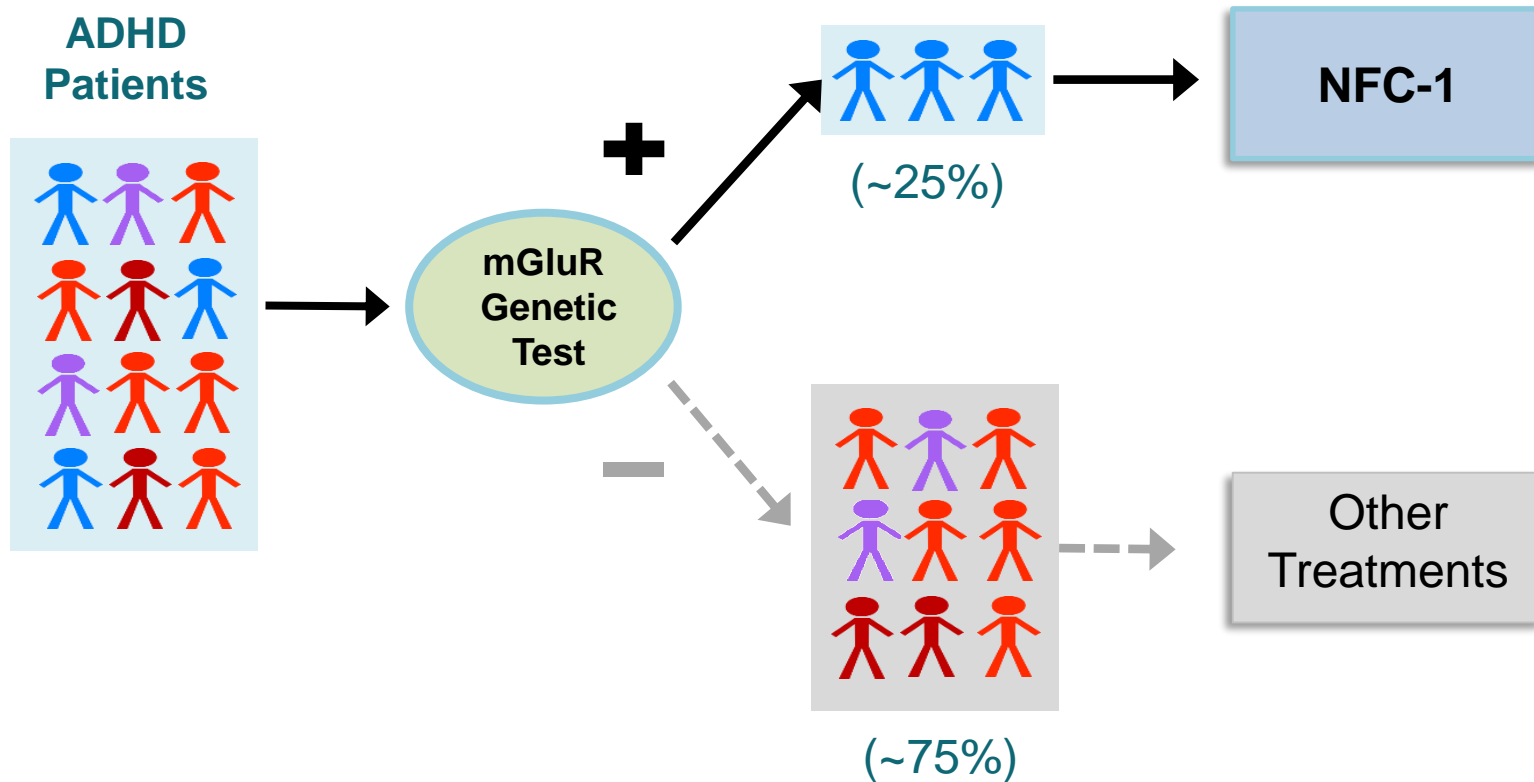
ADHD – Current “Fail First” Treatment Paradigm

- Current diagnostic paradigm is symptom-based without objective biomarkers
- Current treatment paradigm exposes children to multiple stimulants in a “fail first” approach
- Underlying safety concerns create issues with compliance and adherence:
 - Cardiovascular risk
 - Growth retardation
 - Sleep disturbance
 - Anorexia
 - Anxiety
 - Substance abuse
- Average time to discontinuation of medication is 4 months; fully compliant for only 2 months*

*Chacko A et al. Improving medication adherence in chronic pediatric health conditions: a focus on ADHD in youth. *Curr Pharm Des* 2010; 16(22):2416-23.

Genomic Medicine Approach – Changing the Paradigm

Medgenics' approach selectively targets mGluR+ ADHD patients for treatment with NFC-1



Potential for Superior Product Profile

- NFC-1 clinical profile in mGluR+ ADHD patients
 - Effective non-stimulant
 - Non-scheduled
 - No evidence of growth retardation, addiction, sleep disturbance
 - No evidence of cardiovascular risk
 - No expectation for black box warning
- Potential to address co-morbid symptoms (e.g., anxiety, conduct, mood)
- Eliminates need for drug holidays
- Potential for increased compliance and adherence

mGluR+ ADHD: Market Opportunity

- Overall US ADHD Market
 - **2015** Sales in excess of **\$10B***
 - **~6M** pediatric/adolescent patients**
 - Stimulants dominate market: 90+% of total prescriptions
- mGluR+ ADHD Market, ages 6-17
 - **1.5M patients** (~25% mGluR+)
 - **\$2-3B market opportunity** based upon current pricing and compliance/adherence
 - Potential upside for premium pricing with superior product profile

*IBIS World.com

**"Trends in the Parent-Report of Health Care Provider-Diagnosed and Medicated Attention-Deficit/Hyperactivity Disorder: United States, 2003–2011", [Journal of the American Academy of Child & Adolescent Psychiatry, Volume 53, Issue 1, January 2014, Pages 34–46.e2](#)

Financial Update

Q1 2016 Financial Update

- Gross R&D expenses for the 1st Quarter were \$7.0M increasing from \$3.9M for the same period in 2015
 - Due mainly to increased spend on the NFC-1 program and CHOP collaboration
- G&A expenses for the 1st Quarter were \$4.2M increasing from \$3.9M for the same period in 2015
- Cash balance as of March 31, 2016 was \$43.9M which based on current operating plans should be sufficient to fund operations into beginning of Q4 2017

Upcoming Milestones

PROGRAM	TIMING
NFC-1	
Complete enrollment in phenotype/genotype study	Q2 16
Initiate enrollment in Phase 2/3 mGluR+ ADHD Adolescent Trial	Q2 16
Initial top line data	H2 16
Initiate enrollment in Phase 1/2 22q Deletion Syndrome Trial*	Q2 16
Initial open-label responder data	H2 16
TARGET CNS	
Preclinical POC	H2 16

*Pending final CHOP approval

Summary

- Enthusiastic response to genomic medicine approach from physician community in ADHD
- Rapid enrollment into phenotype/genotype study bodes well for initiation of Phase 2/3 trial in June 2016
- Two significant data readouts expected in 2H 16
 - mGluR+ ADHD and 22q Deletion Syndrome (orphan program)
- Anticipate additional programs from the CHOP collaboration in the near-term