



# Corporate Overview

October 2020

# Forward-Looking Statements

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# Zynerba Pharmaceuticals (NASDAQ: ZYNE)

## A Rare/Near-Rare Neuropsychiatric Company





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- Deep pipeline focused on high unmet medical needs; translating into multi-billion dollar market opportunity with Zygel™ (Cannabidiol Gel)
- Currently pursuing four neuropsychiatric indications:
  - Fragile X syndrome (FXS) – pivotal trial complete
  - Developmental and epileptic encephalopathies (DEE) – Phase 2 complete
  - Autism spectrum disorder (ASD) – Phase 2 complete
  - 22q11.2 deletion syndrome (22q) – Phase 2 ongoing
- Experienced team
  - Proven development and commercialization track record in transdermal delivery, orphan diseases, neurology, and psychiatry
- Well capitalized with cash runway expected into 4Q2021
- Multiple expected near term milestones





# Deep Clinical Pipeline

Indication	Preclinical	Phase 1	Phase 2	Pivotal	Expected Milestones
Fragile X Syndrome (FXS)*					<p>Meet with FDA to discuss pivotal results in patients with a fully methylated <i>FMR1</i> gene in 4Q2020; outcome also expected in 4Q2020</p> <p>Communicate specific DEE syndrome(s) for further clinical development around year-end 2020</p> <p>Discuss clinical path forward with FDA in 2H2020</p> <p>Completion of enrollment once COVID-19 restrictions in Australia are eased</p>
	CONNECT-FX: Topline data released				
Developmental and Epileptic Encephalopathies (DEE)					
	BELIEVE: Topline data released				
Autism Spectrum Disorder (ASD)					
	BRIGHT: Topline data released				
22q Deletion Syndrome (22q)					
	INSPIRE: Ongoing				

\*Orphan Drug Designation



# Zygel (ZYN002) Cannabidiol Gel

## Differentiated



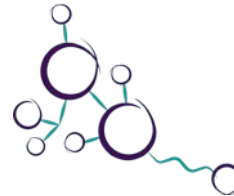
First & only patent-protected (2038), permeation-enhanced, pharmaceutically-produced Cannabidiol gel

## Transdermal

CBD

Formulation delivers Cannabidiol through the epidermis and into the circulatory system

## Unique Mechanism of Action



Cannabidiol modulates multiple receptors and mediates numerous pathways, including the endocannabinoid pathway

## Neuropsych Indications



Potential utility in rare / near-rare neuropsychiatric conditions

**FDA Fast Track and Orphan Drug designations in FXS**

**Orphan Drug designation in 22q**





# Fragile X Syndrome (FXS)

# Fragile X Syndrome (FXS) Overview

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- Rare genetic developmental disability
- Leading known cause of both inherited intellectual disability and autism spectrum disorder
- Symptoms linked to deficiencies in the endocannabinoid system (ECS)
  - System of neurotransmitters regulates emotional responses, behavioral reactivity to context, social interaction
  - Mutation in *FMR1* gene on the X chromosome causes dysregulation of the EC system and results in core cognitive, social, and behavioral symptoms of FXS
- Approximately 40K U.S. patients with a fully methylated *FMR1* gene (FMet)
- No approved drugs indicated for FXS



# Fragile X Syndrome (FXS) Overview



## Full Methylation (FMet) of *FMR1* Gene May be a Biomarker for Disease Impact

- *FMR1* gene codes for production of FMRP\* which is vital to synapse development
- Mutation manifests as multiple repeats of a DNA segment (CGG) in *FMR1*
  - No Fragile X: Segment repeats 5 to 40 times; normal production of FMRP
  - Premutation Fragile X: Segment repeats 50 to 200 times; reduced FMRP production
  - Full mutation FXS: Segment repeats >200 times; usually causes severely impacted or non-functional *FMR1* and leads to core FXS behaviors
- Methylation of *FMR1* also plays a role in determining functionality of the gene
  - At  $\geq 90\%$  methylation (“full methylation” or FMet), *FMR1* is silenced
  - No FMRP is produced: Systems and processes affected by FMRP become dysregulated
- Patients with full mutation FXS and full methylation of *FMR1* are generally the most severely impacted by the disorder: lower IQ, more impacted behaviors
  - ~60% of patients with full mutation FXS are believed to fall into this category

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\*FMR Protein; RNA-binding protein that helps regulate synaptic development and plasticity





# Proposed MOA of Cannabidiol in patients with FXS and fully methylated FMR1 gene



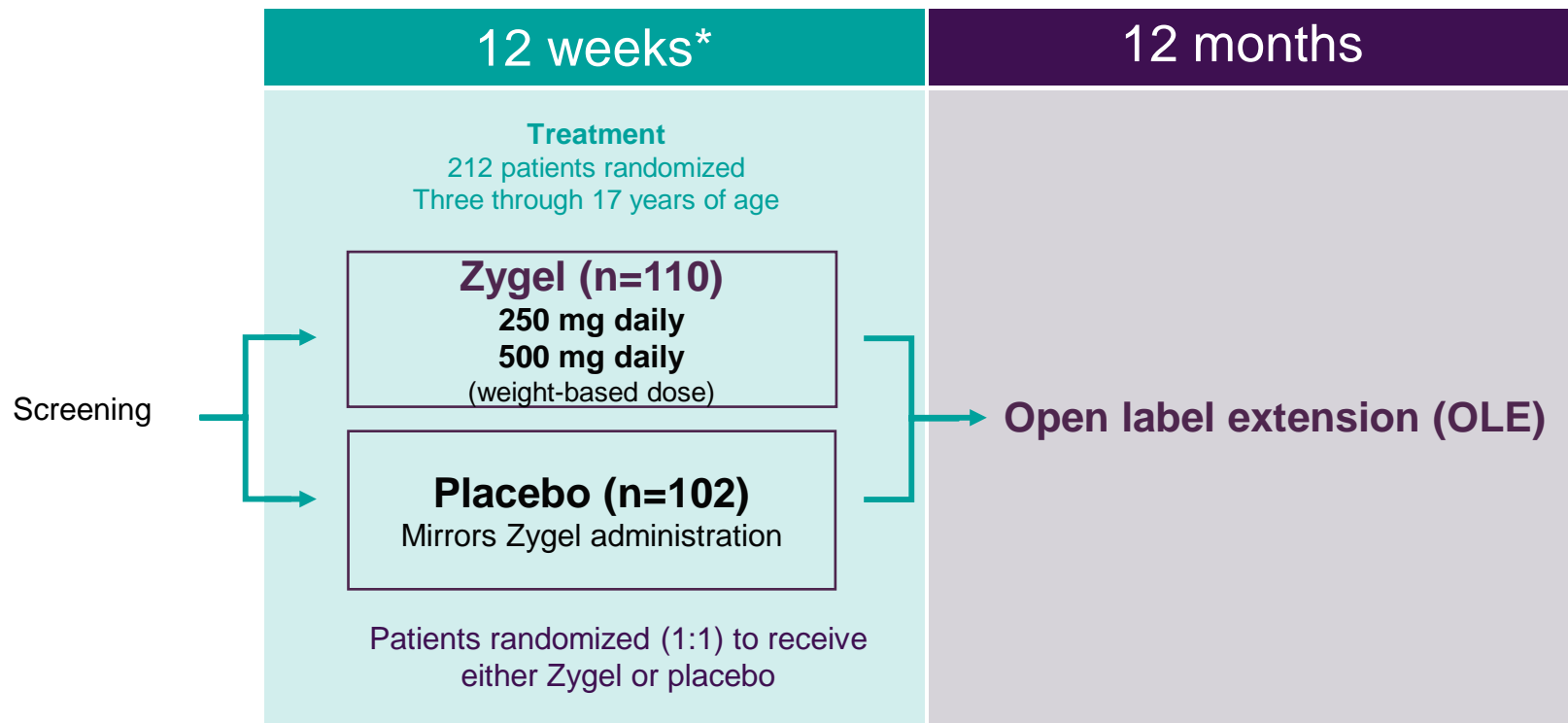
- FMRP affects multiple intracellular processes
  - Endocannabinoid system
  - Glutamate and GABA feedback loops
- FMet patients
  - Males produce no FMRP
  - Females have significant reduction in FMRP
- Absence or loss of FMRP is correlated with behavioral and cognitive deficits
- Cannabidiol in FMet patients
  - Attenuates the disruption of the ECS by increasing the availability of the endocannabinoids (2-AG and anandamide)
  - Modulates the glutamate and GABA feedback loops



# CONNECT-FX: A Pivotal Trial In FXS



Clinical study Of CaNNabidiol in ChildrEn and AdolesCenTs with Fragile X (CONNECT-FX)



\*2 weeks placebo period, followed by 12 weeks treatment.



# CONNECT-FX

## Baseline Characteristics



	Placebo	Zygel	Total
<b>n</b>	102	110	212
<b>Age (years)</b>	9.8	9.6	9.7
<b>Sex – Males, n (%)</b>	78 (76%)	81 (74%)	159 (75%)
<b>Weight (kg)</b>			
<b>Median</b>	34.3	36.8	35.7
<b>Range (Min, Max)</b>	15.6, 104.7	14.6, 87.0	14.6, 104.7
<b>&gt;35kg, %</b>	48%	56%	52%
<b>Baseline psychoactive medications, %</b>	66%	57%	62%

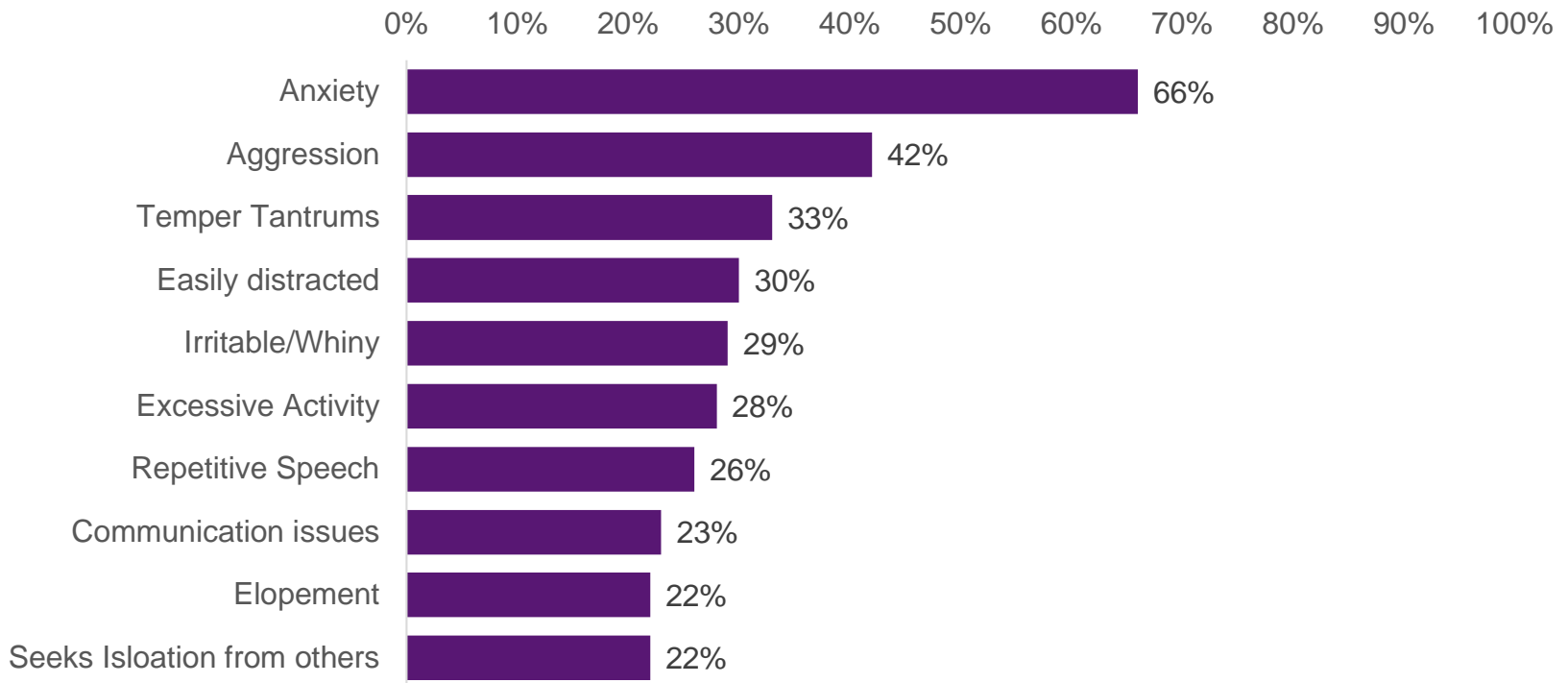


# CONNECT-FX: Qualitative Caregiver Reported Behavioral Survey



Utilizing the Qualitative Caregiver Reported Behavioral Survey, caregivers were asked to describe their most important behavioral challenges at baseline

## Top 10 Classifications of Behavioral Challenges



# CONNECT-FX: A Pivotal Trial In FXS



- Primary endpoint:
  - Change from baseline to end of treatment in ABC-C<sub>FXS</sub> Social Avoidance subscale
- Key secondary endpoints:
  - Change from baseline to end of the treatment in
    - ABC-C<sub>FXS</sub> Irritability subscale score
    - ABC-C<sub>FXS</sub> Socially Unresponsive/Lethargic subscale score
  - Improvement in Clinical Global Impression (CGI-I) at end of treatment, anchored to FXS behaviors
- Aligned with FDA's 'Voice of the Patient' Guidance
  - Captured qualitative data on clinical relevance of FXS behaviors

Zygel did not statistically significantly separate from placebo on the primary or key secondary endpoints in the full analysis set



# CONNECT-FX: Rationale for Pre-Planned Ad Hoc Analysis

## Building on the Scientific Evidence



### Background

- Methylation of genes is considered to be important in numerous pathological disorders including FXS
- Methylation has been associated with the mechanism of mGluR5 in FXS
- Currently, treatment options are limited for many of these disorders
- The degree of methylation can influence the severity of FXS symptoms

### CONNECT-FX

- Pre-planned analysis of the most severely impacted patients defined by patients having  $\geq 90\%$  methylation (“full methylation” or “FMet”) of the impacted *FMR1* gene
  - 80% of the patients enrolled in CONNECT-FX were FMet
- Analysis to explore differences in two groups:
  - FMet group (n=167)
  - Non-FMet group (n=42)



# CONNECT-FX



## Full Data Set, FMet and Non-FMet

### Patient Disposition: FMet Group Comprised 80% of Full Data Set Patients

Patients	Full Data Set	FMet Group	Non-FMet Group
Randomization (ITT)	212	169	42
Full Analysis set	210	167	42

- One patient did not receive study medication after randomization and one patient did not have post-baseline efficacy assessments resulting in 210 patients in Full Analysis set
- One patient with FMR1 gene deletion was not included in either the FMet or Non-FMet groups



# CONNECT-FX: Demographics and Baseline Characteristics



## Similar in the Full Data Set and FMet Group

	Full Data Set Group			FMet Group		
	Placebo	Zygel	Total	Placebo	Zygel	Total
<b>n</b>	102	110	212	77	92	169
<b>Age (years)</b>	9.8	9.6	9.7	9.6	9.2	9.4
<b>Sex – Males (%)</b>	78 (76%)	81 (74%)	159 (75%)	54 (70%)	65 (71%)	119 (70%)
<b>Weight (kg)</b>						
<b>Median</b>	34.3	36.8	35.7	33.9	35.7	35.0
<b>Range (Min, Max)</b>	15.6, 104.7	14.6, 87.0	14.6, 104.7	15.6, 104.7	14.6, 87.0	14.6, 104.7
<b>&gt;35kg, %</b>	48%	56%	52%	46%	53%	50%
<b>Baseline psychoactive medications, %</b>	66%	57%	62%	65%	54%	59%





# Pre-Planned Ad Hoc Results: FMet Group



Zygel Achieved Statistical Significance on Social Avoidance: Changes From Baseline to Week 12 (ABC-C<sub>FXS</sub>)

		Placebo N=76			Zygel N=91					
Endpoints		Baseline Mean (SE)	Week 12 Mean (SE)	Week 12 Median Percent Change	Baseline Mean (SE)	Week 12 Mean (SE)	Week 12 Median Percent Change	Treatment Difference / Odds Ratio <sup>†</sup>	Treatment <i>p</i> -value	
Primary Endpoint	Social Avoidance	7.18 (0.32)	5.41 (0.42)	-21.1	7.12 (0.29)	4.32 (0.33)	-40.0	-1.00	<b>0.020*</b>	
	Secondary Endpoints	Irritability	28.0 (1.56)	24.11 (1.56)	-11.6	29.36 (1.37)	22.69 (1.42)	-24.3	-2.30	0.091
		Socially Unresponsive /Lethargic	13.17 (0.85)	10.29 (0.80)	-20.5	13.30 (0.68)	9.03 (0.67)	-30.8	-1.17	0.135
		CGI-I	-	35.7%		-	51.1%		1.88 <sup>†</sup>	0.056

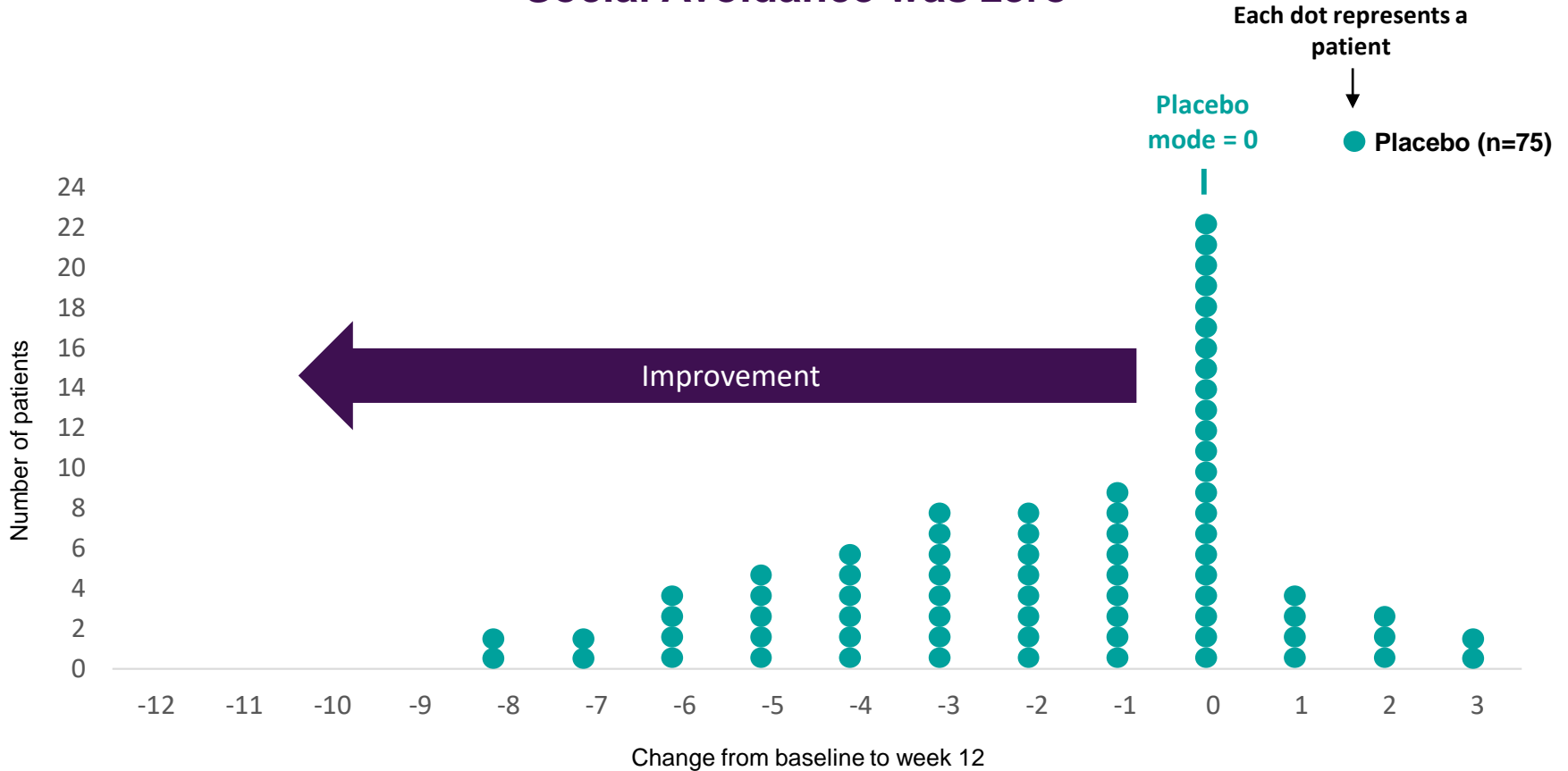
\*Statistically significant



# CONNECT-FX: ABC-C<sub>FXS</sub> Social Avoidance Changes From Baseline to Week 12 in FMet Group



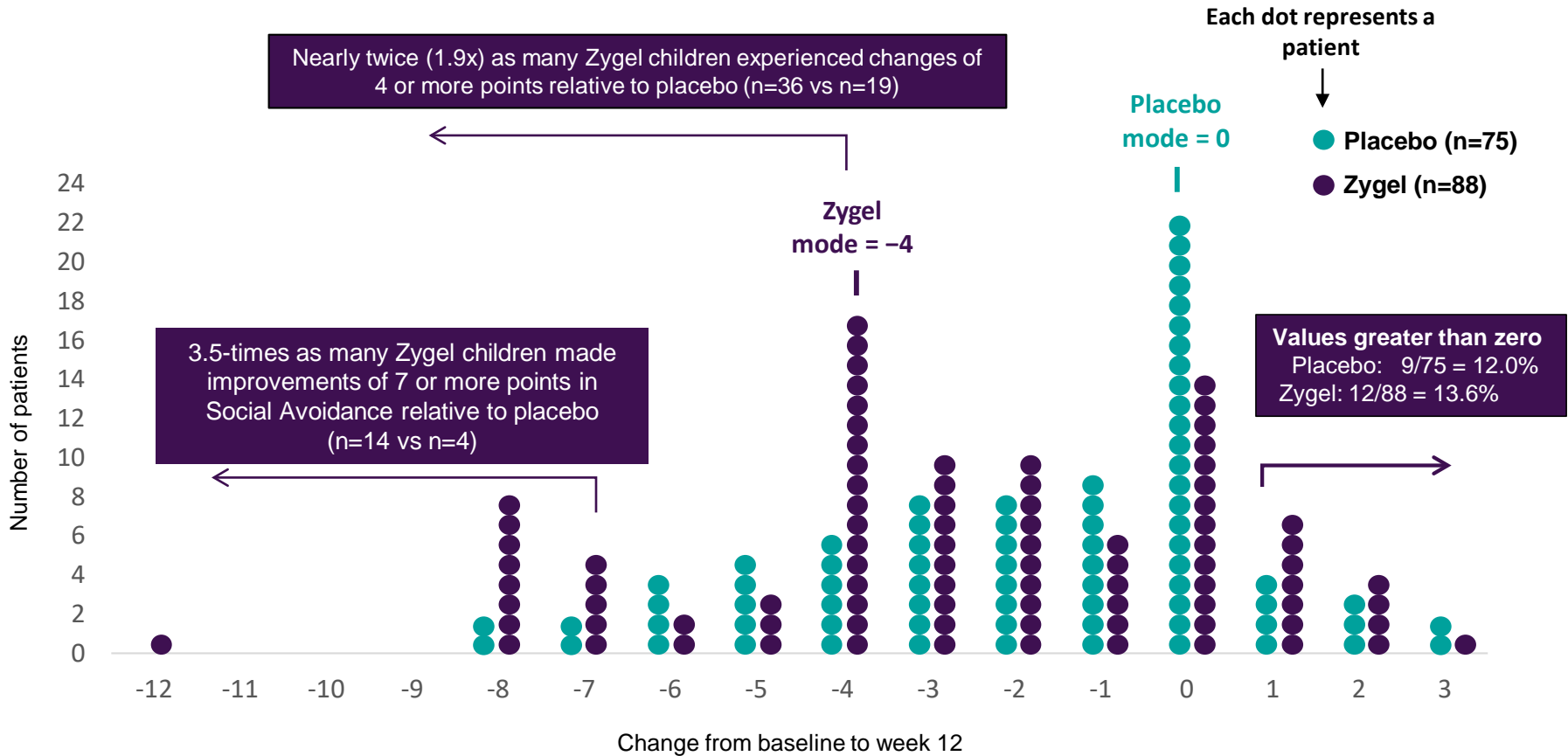
In placebo group, the most common change (mode) in Social Avoidance was zero



# CONNECT-FX: ABC-C<sub>FXS</sub> Social Avoidance Changes From Baseline to Week 12 in FMet Group



## Zygel group demonstrated greater improvement versus placebo



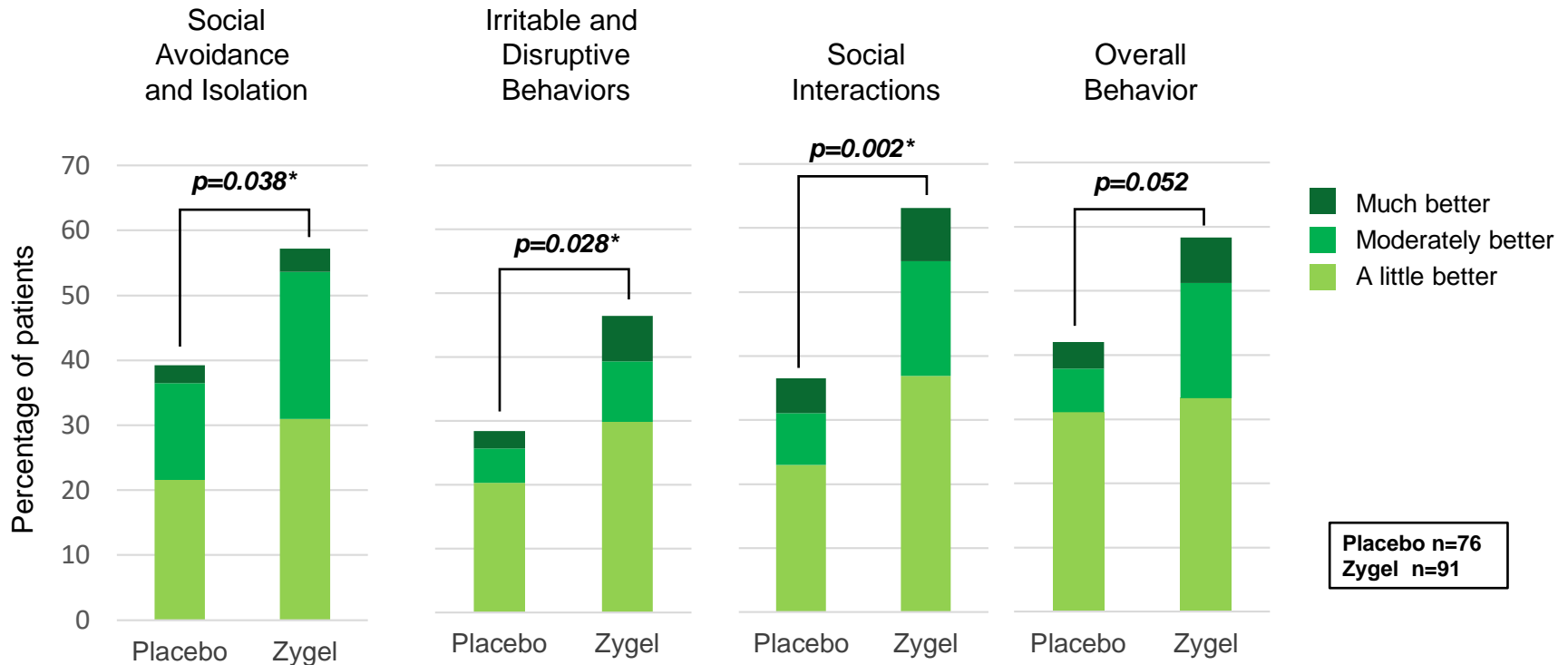
Data represent observed cases: 4 patients did not have Week-12 ABC-C<sub>FXS</sub> assessment



# CONNECT-FX: Caregiver Global Impression-Change: FMet Group



## Change from Baseline to Week 12: Broad Shifts Towards Global Improvement



\*Statistically significant

P-values indicate "betterment" on Zygel vs "betterment" on placebo



# Psychometric Analyses Determined Clinically Meaningful Changes for ABC-C<sub>FXS</sub>

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- Clinically meaningful treatment responses over 12 weeks of treatment determined to be an improvement of:
  - Three (3) points for the Social Avoidance subscale
  - Nine (9) points for the Irritability subscale
  - Five (5) points for the Socially Unresponsive / Lethargic subscale

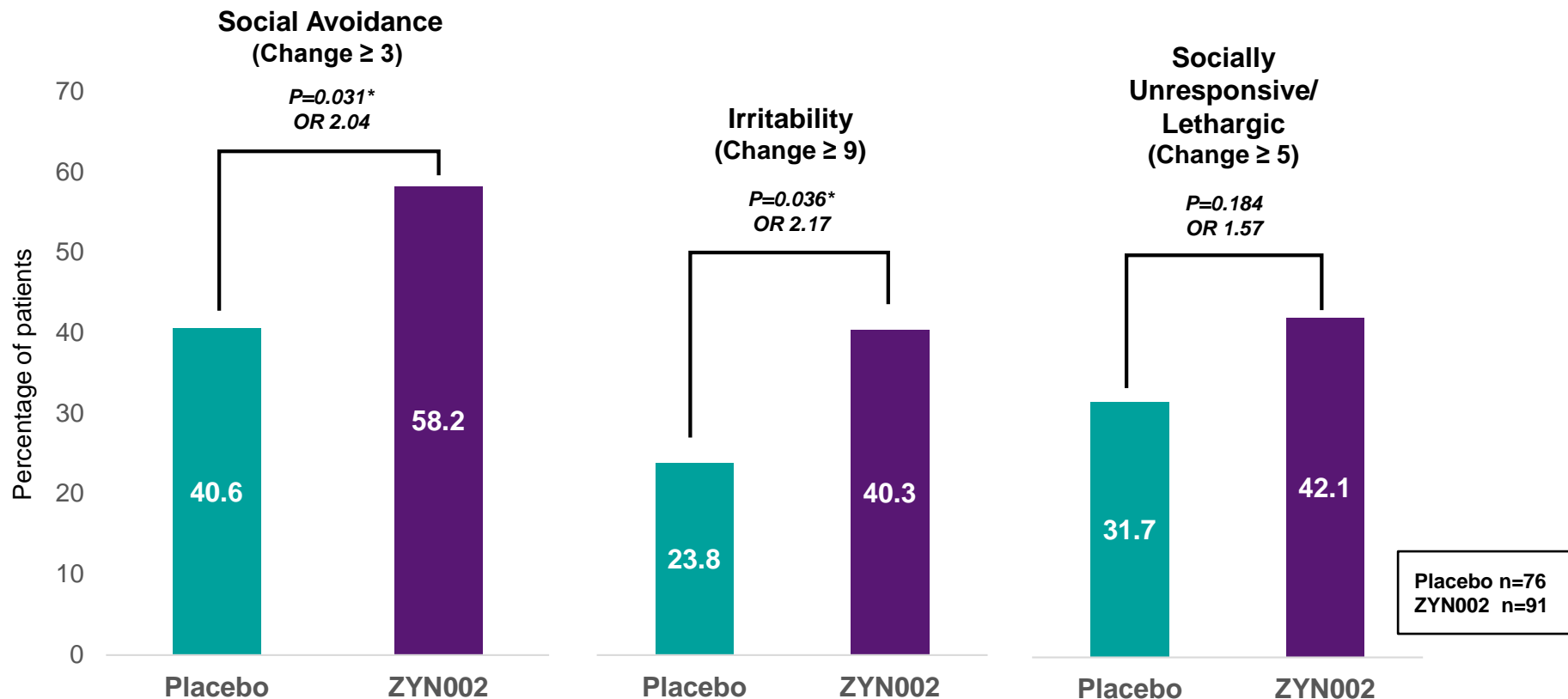
Data presented at the Joint 16th International Child Neurology Congress (ICNC) & 49th Annual Child Neurology Society (CNS) Meeting, Oct. 15, 2020



# FMet Patients With Clinically Meaningful Change in ABC-C<sub>FXS</sub> Subscales



Zygel achieved statistically significant separation from placebo in Social Avoidance & Irritability subscales



Data presented at the Joint 16th International Child Neurology Congress (ICNC) & 49th Annual Child Neurology Society (CNS) Meeting, Oct. 15, 2020



# Zygel Advantages Observed in Social Avoidance Are Supported by Caregiver and Clinical Global Impression Improvements in FMet Group



## Primary Endpoint: ABC-C<sub>FXS</sub> Social Avoidance Subscale

- Statistically significantly improvement vs. placebo (p=0.020\*)

## Caregiver Global Impression – Change at Week 12 (Four Domains)

- Statistically significant improvement vs. placebo in Social Interactions (p=0.002\*)
- Statistically significant improvement vs. placebo in Irritable & Disruptive Behaviors (p=0.028\*)
- Statistically significant improvement vs. placebo in Social Avoidance & Isolation (p=0.038\*)
- Trend toward statistical significance in improvement in Overall Behavior vs. placebo (p=0.052)

## Clinical Global Impression - Improvement\*\* (anchored to FXS behaviors; clinician rated)

- Trend toward statistical significance vs. placebo (p=0.056)

## Clinically Meaningful Improvements in ABC-C<sub>FXS</sub> Subscales at Week 12

- Significantly more patients achieved a clinically meaningful change in ABC-C<sub>FXS</sub> Social Avoidance (≥3 points; p=0.031\*) and Irritability (≥9 points; P=0.036\*) with Zygel vs. Placebo

\* Statistically significant

\*\* Not specific to Social Avoidance



# CONNECT-FX: Safety

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- Zygel was very well tolerated
  - Safety profile consistent with previously released data from all other Zygel trials
  - No safety signal identified
- No serious or severe adverse events reported during the study
- All treatment-emergent adverse events (TEAEs; any event, whether unrelated or related to study drug) were mild or moderate
  - Most common treatment-related TEAE: application site pain
    - Zygel: 6.4%; placebo: 1.0%
  - Seven total psychiatric disorder TEAEs; 5 were in placebo group
- Laboratory values for chemistry and hematology comparable between placebo and Zygel groups; no clinically relevant abnormalities in either group
  - No clinically significant changes to liver function tests





# Next Steps in Fragile X Program

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- Meeting with FDA to discuss CONNECT-FX data and the regulatory path forward in FMet patients scheduled for 4Q2020
  - Zynerva also expects to disclose the outcome of the meeting in 4Q2020
- Pivotal data to be published and presented at upcoming medical meetings





# Autism Spectrum Disorder (ASD) in children and adolescents



# ASD in Pediatrics Overview

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- Near-rare disorder affecting ~1MM pediatric and adolescent patients
- Symptoms include
  - Irritability
  - Anxiety
  - Restricted, repetitive patterns of behavior
  - Impairments in social communication
  - Deficits in verbal and non-verbal communication
  - Deficits in developing, understanding and maintaining relationships
- Most diagnosed after age 4; can be diagnosed as early as age 2
- Significant unmet medical need
  - Accelerating rate of diagnosis but only two FDA approved products
    - Both atypical antipsychotics have significant side effect profile
    - Neither approved to address the key symptoms of social impairment and anxiety



# Rationale for Developing Zygel in ASD

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- Newer studies suggest ASD is linked to disruption in the EC system
  - Altered anandamide signaling may contribute to ASD-related social and communication impairments
  - EC system modulates many cellular functions and molecular pathways altered in ASD: imbalanced GABAergic, glutamatergic transmission, oxidative stress, immune dysregulation and altered energy metabolism
- Clinical and anecdotal data demonstrate that children dosed with Cannabidiol displayed an improvement in social avoidance and anxiety
  - May modulate the EC system and improve certain autism-related behaviors
- Two recent US patents directed to methods of treating ASD by transdermally administering synthetic or purified cannabidiol, respectively, provide IP protection to 2038

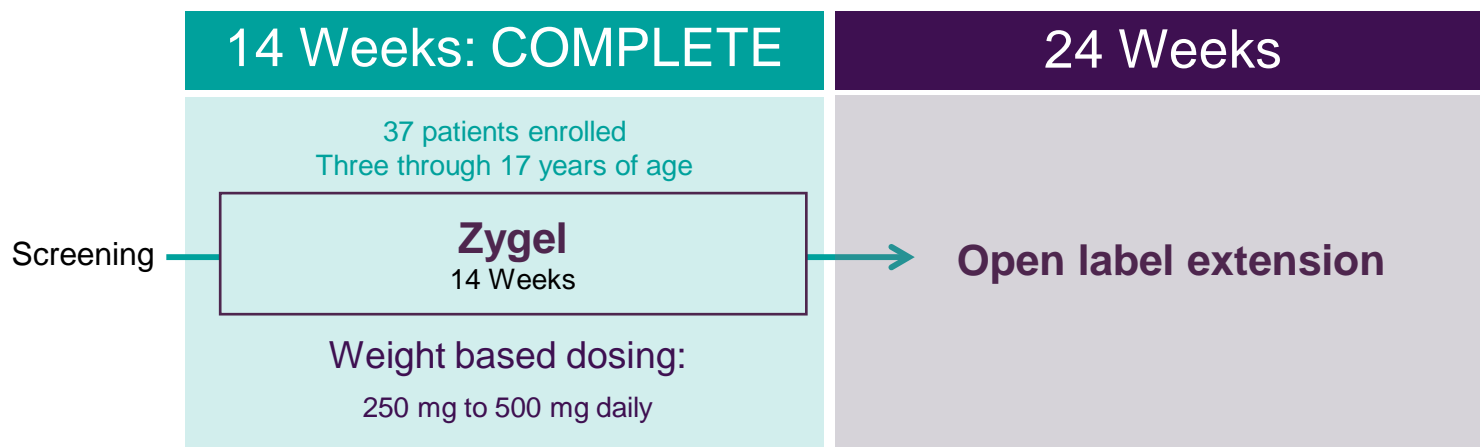




# BRIGHT Phase 2 Trial in ASD

Positive Topline Data Reported on May 27, 2020

Open-Label Tolerability and Efficacy Study of ZYN002 Administered as a Transdermal Gel to Children and Adolescents with Autism Spectrum Disorder



Efficacy assessments (primary efficacy assessment = week 14 vs baseline) :

- Aberrant Behavior Checklist (ABC-C)
- Parent Rated Anxiety Scale – Autism Spectrum Disorder (PRAS-ASD)
- Autism Parenting Stress Index
- Autism Impact Measure (AIM)
- Clinical Global Impression – Improvement (CGI-I) and Severity (CGI-S)
- Qualitative Caregiver Reported Behavioral Problems Survey



# BRIGHT Trial Patient Populations



Baseline Patient Population: BRIGHT	
Patients enrolled (n)	37
Included in safety analysis	37
Included in efficacy analyses	36*
Discontinuations	9
Patients completing 14-week trial	28

\* One patient was lost to follow up and did not have post-dosing efficacy assessments



# BRIGHT Trial Patient Demographics



Baseline Patient Demographics	
Patients enrolled, n	37
Age, years Mean (range)	9.2 (3-16)
Sex, n (%) Male Female	34 (91.9%) 3 (8.1%)
Race, % White Asian Native Hawaiian or other Pacific Island Other	70.3% 8.1% 2.7% 18.9%
Time to diagnosis, years	5.4
Underlying medication, % Subjects entering with $\geq 1$ underlying medication Subjects entering with $\geq 1$ underlying psychotropic medication (includes anti- depressants, anxiolytics and antipsychotics)	92% 65%



# Strong Safety and Tolerability Profile in BRIGHT Trial in ASD

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- Well tolerated; consistent with previously released data
- Fewer than half of patients experienced an adverse event (AE); most were mild and transient
- Only 14% of patients experienced a treatment-related AE
  - All application site-related
- No severe or serious AEs reported during the study.

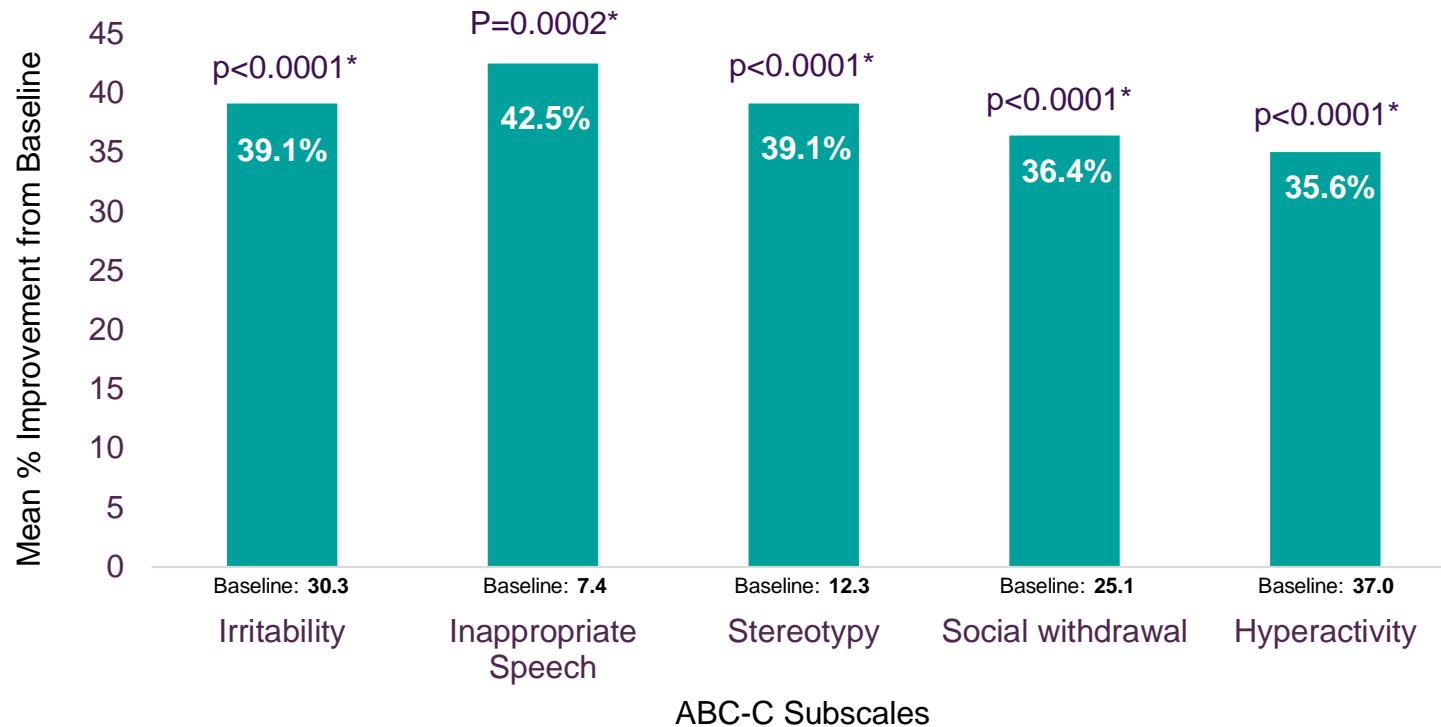




# Percent Improvement in ABC-C Subscale Scores at Week 14 vs. Baseline



Statistical Significance Achieved in All Subscales



\* Statistically significant



# ABC-C Responses Supported by Other Efficacy Assessments

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Results of other efficacy assessments support the results demonstrated in the ABC-C, including:

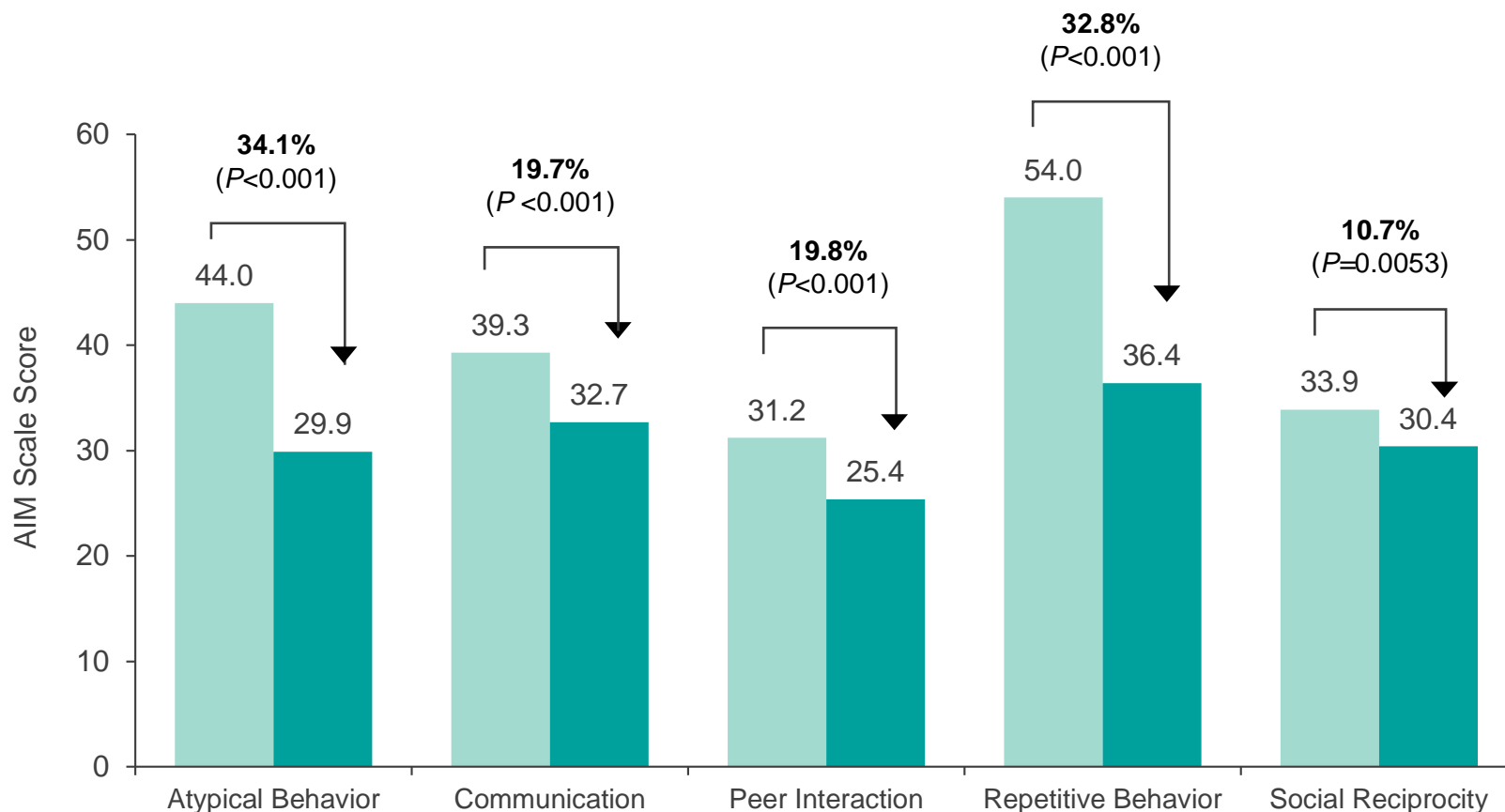
- Parent Rated Anxiety Scale - Autism Spectrum Disorder (PRAS-ASD):
  - Mean improvement of 46% at week 14 from baseline ( $p < 0.0001$ )
- Clinical Global Impression - Improvement (CGI-I)
  - 57% of patients were rated by clinician as Very Much or Much Improved at week 14
- Zynerva intends to present additional data at future medical meetings



# Statistically Significant Improvement in Autism Impact Measure Scores



## Phase 2 BRIGHT Trial Autism Impact Measure (AIM)



Data presented at the Joint 16th International Child Neurology Congress (ICNC) & 49th Annual Child Neurology Society (CNS) Meeting, Oct. 15, 2020

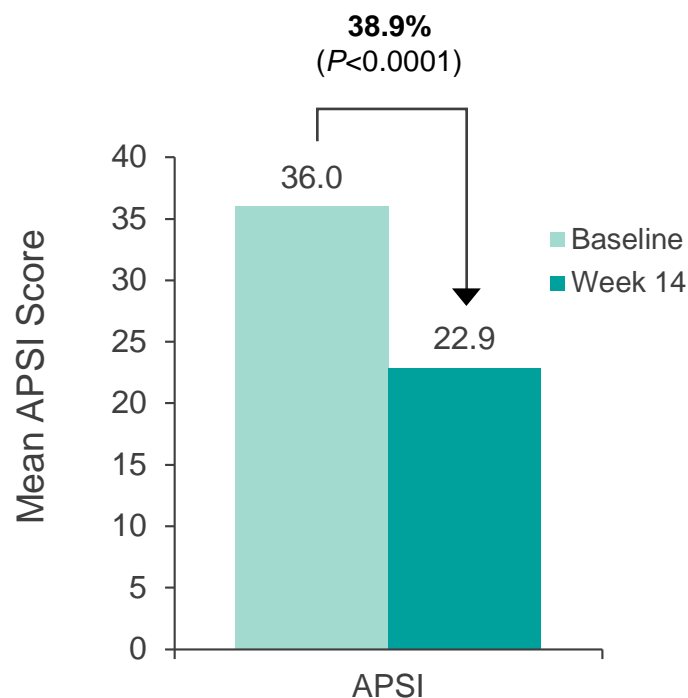
■ Baseline  
■ Week 14



# Statistically Significant Improvement in Autism Parenting Stress Index

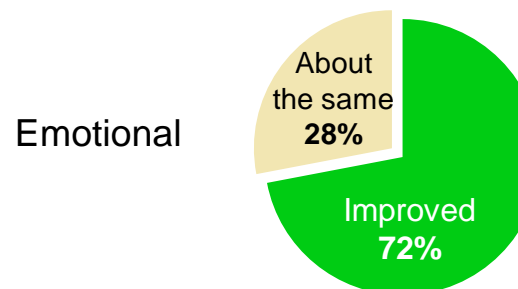
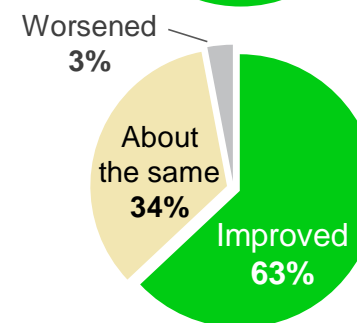
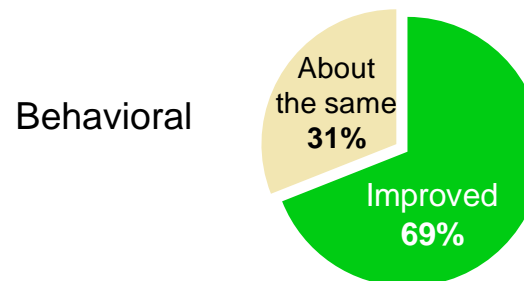


## Phase 2 BRIGHT Trial Autism Parenting Stress Index



Data presented at the Joint 16th International Child Neurology Congress (ICNC) & 49th Annual Child Neurology Society (CNS) Meeting, Oct. 15, 2020

## Additional improvements in the Qualitative Caregiver Behavioral Problems Survey





# 22q11.2 Deletion Syndrome (22q)

# 22q Overview

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- Most common contiguous gene deletion syndrome
- Rare disorder: ~81K patients in US
- Midline condition with abnormalities affecting palate, face, heart and other organs; surgically corrected in infancy
- Neuropsychiatric illnesses (anxiety disorders, ASD) and learning disabilities common and impactful
  - 22q associated with increased anxiety, withdrawn behavior and social interaction problems
  - Early onset of neuropsychiatric symptoms disrupts development and quality of life, and heightens risk of later psychotic disorders
    - 25-fold increased risk of developing schizophrenia vs. 1% lifetime risk in general population



# 22q Patient Management

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- Two primary stages of 22q patient management:
  - During infancy, doctors address acute physical concerns, such as anomalies of heart and palate, with surgery
  - Once the physical concerns are stabilized, focus shifts to managing neuropsychiatric symptoms, such as anxiety and autistic behaviors
- No currently approved drugs indicated for 22q



# Rationale for Developing Zygel in 22q

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- Cannabidiol may treat neuropsychiatric symptoms in 22q due to activity as:
  - Modulator of endocannabinoid system
  - Agonist at serotonin<sub>1A</sub> receptors
  - Antagonist at GPR55 receptors
- Early control of anxiety may delay the development of psychosis
- Phase 2 study underway in pediatric and adolescent patients with 22q
  - Enrollment delayed due to COVID-19 travel restrictions in Australia; topline results timeline to be announced following lifting of restrictions
- Orphan Drug designation received for Cannabidiol for the treatment of 22q



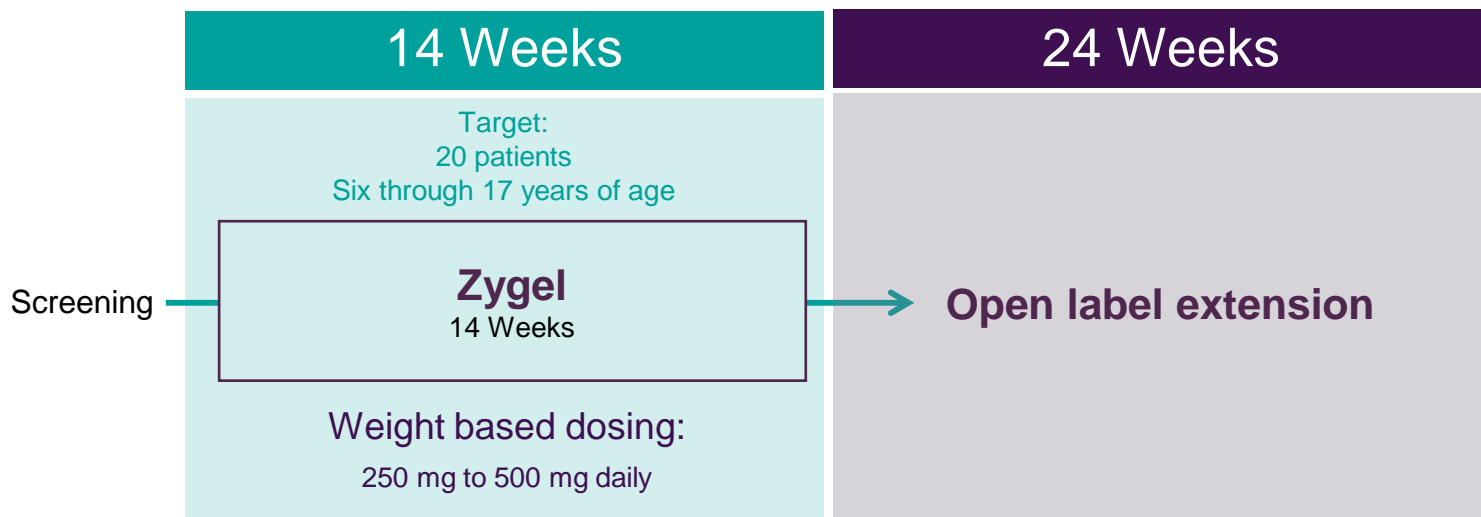




# INSPIRE Phase 2 Trial in 22q

## Enrollment Ongoing

Assessing the **I**mpact of **Zygel** (Transdermal Cannabidiol Gel) on **P**ediatric **B**ehavioral and **E**motional Symptoms of 22q11.2 Deletion Syndrome



Efficacy assessments (week 14 vs baseline) include:

- Aberrant Behavior Checklist-Community (ABC-C)
- Anxiety, Depression and Mood Scale (ADAMS)
- Qualitative Caregiver Reported Behavioral Problem Survey
- Clinical Global Impression – Severity and Improvement





# DEE

## Developmental and Epileptic Encephalopathies

# DEE Patients are Medically Fragile

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- Group of rare / ultra rare childhood-onset epilepsies with impaired or regressed developmental progress
- Cognitive impairment, psychiatric problems, and behavioral disturbances are phenotypic
- Medically fragile population
  - Comorbidities include cerebral palsy, chronic respiratory infections, gait disturbances, movement disorders, scoliosis, and feeding problems
  - Includes wheelchair bound individuals with feeding tubes
- Most common and debilitating seizure types in DEEs are:
  - Focal impaired-awareness seizures (FIAS) – formerly known as complex partial
  - Focal to bilateral tonic-clonic and generalized tonic-clonic seizures (TCS) – commonly known as convulsive seizures (CS)

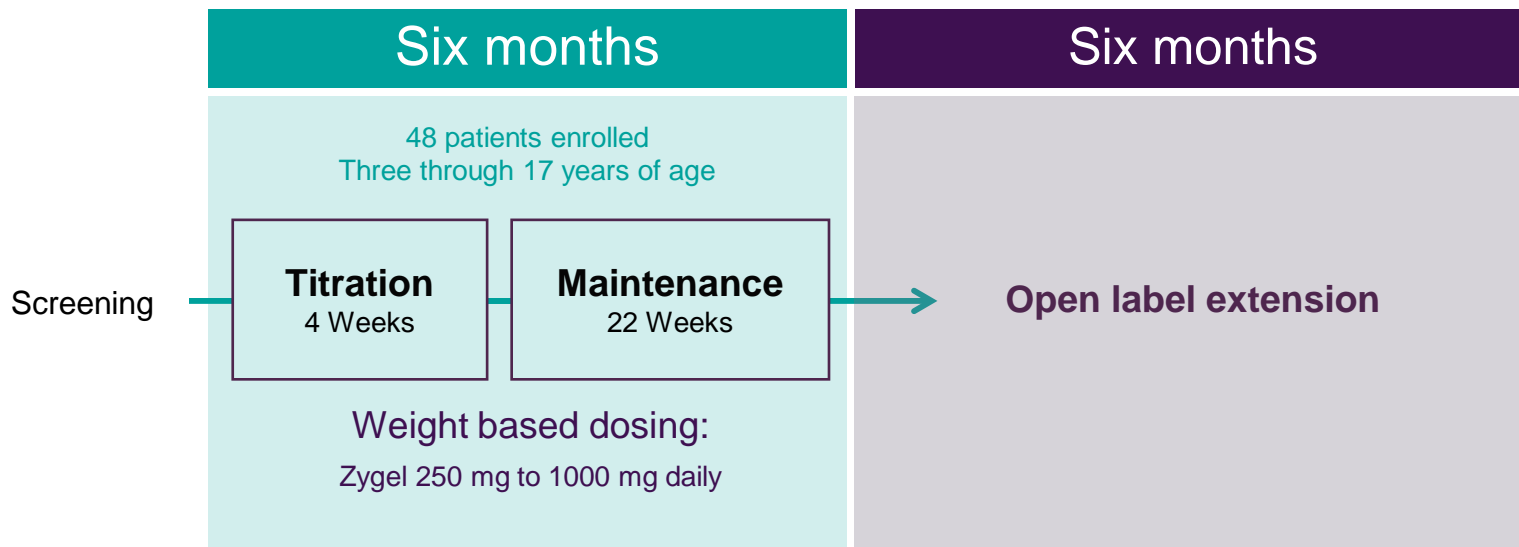




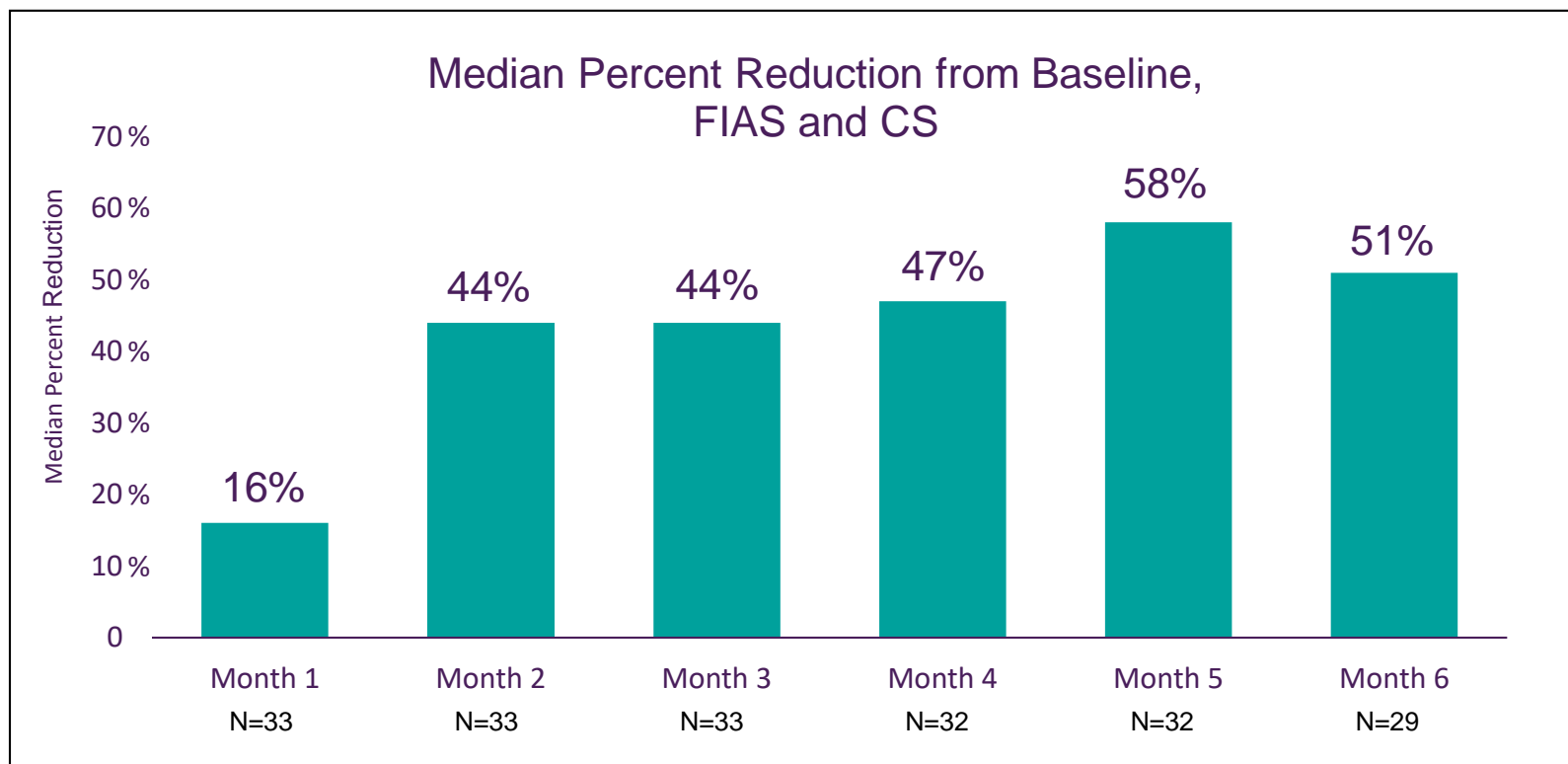
# BELIEVE Phase 2 Trial in DEE

Completed; Reported Positive Topline Results on 9/18/19

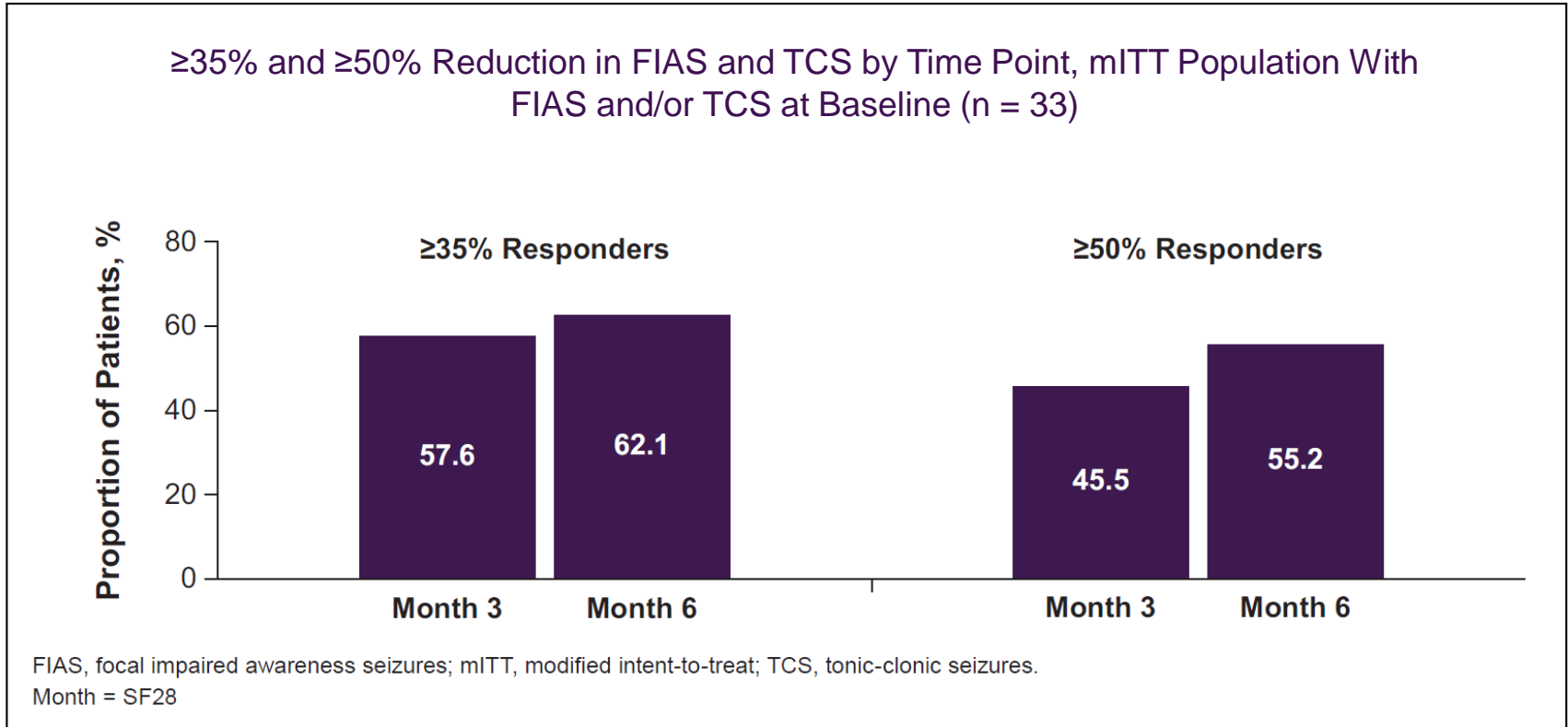
Open LaBel Study to Assess the Safety and Efficacy of ZYN002 Administered as a Transdermal Gel to Children and Adolescents with Developmental and Epileptic Encephalopathy



# BELIEVE: Clinically Meaningful Seizure Reductions from Baseline and Sustained through Six Months in DEE



# BELIEVE: Percentage of Patients with $\geq 35\%$ and $\geq 50\%$ Reduction in FIAS and TCS



Data reported at the 2020 American Academy of Neurology (AAN) Science Highlights Virtual Session



# BELIEVE Safety



## Zygel Well Tolerated in this Six Month Trial: No Safety Signal Identified

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- All events in six month period, whether unrelated or related to study drug, reported as adverse AEs (e.g.: influenza, runny nose, scrapes, etc.)
- As a result and as anticipated, most patients experienced an AE
  - Most were mild and transient
  - Only one patient discontinued due to an AE (application site reaction)
- Most common treatment-related adverse events occurred in only four patients each:
  - Application site dryness, application site pain, and somnolence (all four patients exhibiting somnolence were taking concomitant clobazam)
- Low rate of serious adverse events (SAEs)
  - Only two SAEs deemed possibly drug-related (LRTI and status epilepticus)
  - No drug-related hepatic, gastrointestinal, or lethargy-related SAEs
- Tolerability profile consistent with the safety database for Zygel



# BELIEVE: Qualitative Assessments of Behavioral and Cognitive Improvements



- Epilepsy and Learning Disabilities Quality of Life (ELDQOL) scale
  - Statistically significant reductions from baseline in subscale scores for seizure severity, behavior, and mood observed at month 6 ( $p < 0.01$ )
- Qualitative caregiver feedback on improvements included:
  - Any improvement: 84% (n = 36)
  - Improved vitality: 58% (n = 25)
  - Improvement in seizures: 51% (n = 22)
  - Improved cognition/concentration: 47% (n = 20)
  - Improved socially avoidant behaviors: 44% (n = 19)
  - Improvement in irritability: 33% (n = 14)
  - School improvement: 28% (n = 12)
  - Medical improvement: 14% (n = 6)

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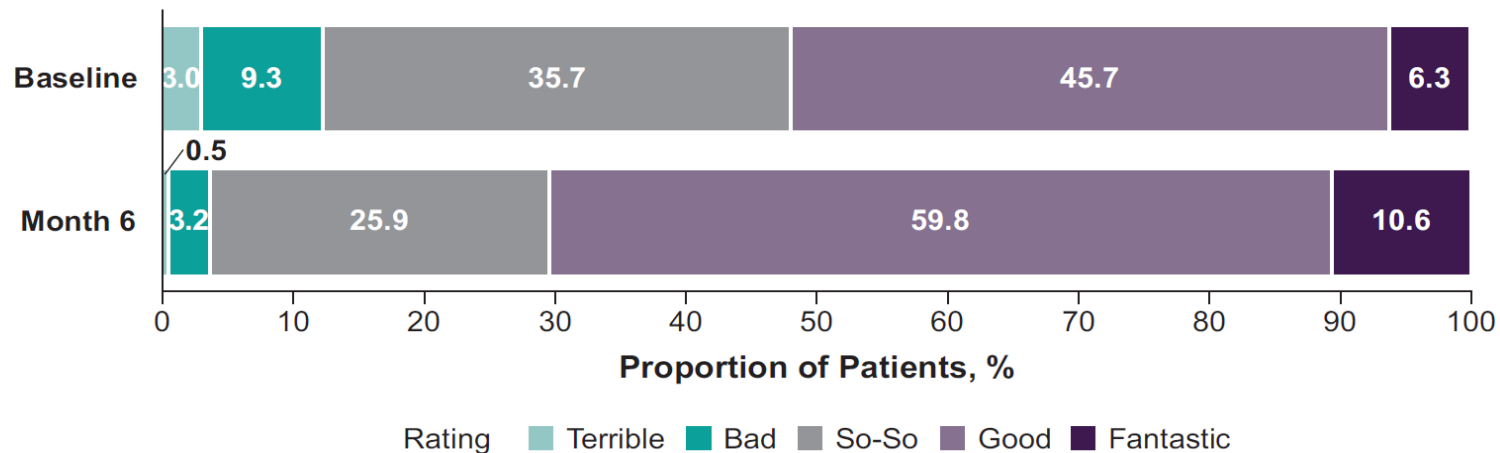




# BELIEVE: Qualitative Assessments of Behavioral and Cognitive Improvements



- Good Day/Bad Day comparing baseline to month six:
  - “Good day” and “fantastic day” reports increased from 52% to 70%
  - “Terrible day” and “bad day” reports decreased from 12% to 4%



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# Compelling Results Suggest a Potential Pathway to Pivotal Trials



- Efficacy results:
  - Clinically meaningful reductions in seizures beginning in month two and sustained through six months
  - Suggest improvements on important behavioral symptoms
- Safety results:
  - Zysel was well tolerated
  - Consistent with previously reported Zysel studies
- The FDA supports a development program which would treat focal-impaired awareness and convulsive seizures
- Due to the heterogeneity of DEE patients, FDA suggests pursuing individual syndromes rather than considering DEE as a single condition
- The Company is evaluating specific DEE syndromes as possible clinical targets; expect to provide update around year-end 2020







# Financial Strength

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- Clean balance sheet
  - No debt, 29.3 M shares outstanding (as of August 6, 2020)
- Cash and cash equivalent position of \$77.0M as of June 30, 2020
- Cash runway expected to be sufficient to fund operations and capital requirements into the fourth quarter of 2021



# Expected Clinical Milestones in 2020

	1Q 2020	2Q 2020	3Q 2020	4Q 2020
 <b>FXS</b>		<input checked="" type="checkbox"/> Report pivotal CONNECT-FX topline results		Discuss results in FMet pts with FDA & disclose outcome
 <b>DEE</b>			<input checked="" type="checkbox"/> Results of FDA discussions on clinical path	Provide update on path forward around Y/E 2020
 <b>ASD</b>		<input checked="" type="checkbox"/> Report Ph. 2 BRIGHT topline results	Discuss clinical path forward in ASD with FDA	
 <b>22q</b>	Enrollment delayed due to COVID-19 travel restrictions in Australia. Topline results timeline to be determined following lifting of restrictions			





# Corporate Overview

October 2020