Forward-Looking Statements

Zogenix cautions you that statements included in this press release that are not a description of historical facts are forward-looking statements. Words such as “believes,” “anticipates,” “plans,” “expects,” “indicates,” “will,” “intends,” “potential,” “suggests,” “assuming,” “designed,” and similar expressions are intended to identify forward-looking statements. These statements include timing of commercial launch of FINTEPLA for the treatment of Dravet syndrome in additional countries in Europe, including France; Zogenix’s expectations on the submission of a J-NDA by Nippon Shinyaku in Japan; the timing and ability of Zogenix to complete regulatory submissions in the United States and the European Union for FINTEPLA in LGS; Zogenix’s plans to expand FINTEPLA in other indications including the timing or success of a Phase 3 clinical trial in CDKL5 deficiency disorder and investigator-initiated clinical trials in other indications; Zogenix’s belief that the recent Type B meeting with the FDA supports an NDA submission for MT1621 in TK2 deficiency and the timing of such submission. These statements are based on Zogenix’s current beliefs and expectations. The inclusion of forward-looking statements should not be regarded as a representation by Zogenix that any of its plans will be achieved. Actual results may differ from those set forth in this release due to the risks and uncertainties inherent in Zogenix’s business, including, without limitation: Zogenix may not be successful in executing its sales and marketing strategy for the commercialization of FINTEPLA in the U.S. and Europe, including due to the costs and procedures related to the REMS certification process or controlled access program; the COVID-19 pandemic may disrupt Zogenix’s business operations, impairing the ability to commercialize FINTEPLA and may delay Zogenix’s development plans for FINTEPLA and MT1621; unexpected adverse side effects or inadequate therapeutic efficacy of FINTEPLA or MT1621 that could limit development or commercialization, or that could result in recalls or product liability claims; additional data from Zogenix’s ongoing studies may contradict or undermine the data previously reported; and other risks described in Zogenix’s prior press releases as well as in public periodic filings with the U.S. Securities & Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof, and Zogenix undertakes no obligation to revise or update this press release to reflect events or circumstances after the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.
About Zogenix

A global pharmaceutical company focused on serious rare diseases

- **Fintepla**® (fenfluramine) lead product approved in the U.S. and Europe for the treatment of Dravet syndrome and under development for the treatment of seizures associated with rare, catastrophic, difficult-to-treat epilepsies

- **MT1621** product candidate in late-stage development for TK2d, a rare, often fatal mitochondrial deficiency disease

- Headquarters in the San Francisco Bay Area

- Additional operations in Europe; partnership in Japan

- 200+ employees

- ~$505M cash and marketable securities on December 31, 2020
# Zogenix Pipeline

Committed to transforming the lives of patients and families living with rare disease

<table>
<thead>
<tr>
<th>Project &amp; Indication</th>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Regulatory</th>
<th>Commercialization</th>
</tr>
</thead>
<tbody>
<tr>
<td>Rare, Treatment-Resistant Epilepsies</td>
<td></td>
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<tr>
<td>Dravet Syndrome – United States</td>
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<td>FDA Approved</td>
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<td>Dravet Syndrome – Europe</td>
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<tr>
<td>Dravet Syndrome – Japan</td>
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<td>Positive Phase 3 Trial</td>
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<tr>
<td>Lennox-Gastaut Syndrome</td>
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<td>Positive Phase 3 Trial</td>
<td></td>
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<tr>
<td>CDKL5 Deficiency Disorder</td>
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<td></td>
<td>Phase 3 Trial</td>
<td></td>
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<tr>
<td>Other Rare Epilepsies</td>
<td></td>
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<td></td>
<td>Investigator-Initiated Trials</td>
<td></td>
</tr>
</tbody>
</table>

**Mitochondrial Disease**

| MT1621                                |             |         |         |         |            |                  |
| TK2d (Thymidine Kinase 2 Deficiency)   |             |         |         |         |            | Positive Efficacy Trial |

**Fintepla® (fenfluramine) oral solution.**
# Market Opportunities

<table>
<thead>
<tr>
<th>Dravet Syndrome</th>
<th>Lennox Gastaut Syndrome</th>
<th>CDKL5 Deficiency Disorder</th>
<th>TK2d Deficiency</th>
</tr>
</thead>
<tbody>
<tr>
<td>~12k-15k</td>
<td>~60k-100k</td>
<td>~8k-10k</td>
<td>~1-2k</td>
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<tr>
<td>US, EU, JPN prevalence</td>
<td>US, EU, JPN prevalence</td>
<td>US, EU, JPN prevalence</td>
<td>US, EU, JPN prevalence</td>
</tr>
<tr>
<td>&gt;80% of patients remain uncontrolled on existing AED regimens</td>
<td>Typical childhood onset (2-7 yrs.) with varied disease etiology</td>
<td>Disease onset at birth, continues throughout life</td>
<td>~50% of patients are infantile onset, with life expectancy of 1-3 years</td>
</tr>
<tr>
<td>Vast majority of patients on multi-drug treatment regimens of 2-5 AEDs</td>
<td>Vast majority of patients on multi-drug treatment regimens of 2-5 AEDs</td>
<td>&gt;70% of patients experience daily seizures</td>
<td>Profound loss/impairment of mobility, respiratory, eating, other functions</td>
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<tr>
<td>Premature childhood mortality, primarily SUDEP, of ~20%</td>
<td>Currently 0 approved therapies</td>
<td>Currently 0 approved therapies</td>
<td>Currently 0 approved therapies</td>
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<tr>
<td>Unprecedented sustained seizure reduction</td>
<td>First-in-class mechanism; highly effective reduction in severe, convulsive seizures</td>
<td>Initial open-label efficacy in convulsive seizures similar to results seen in Dravet</td>
<td>MT-1621</td>
</tr>
<tr>
<td>New potential transformational, disease-modifying therapy</td>
<td>&gt;$1B Opportunity</td>
<td>&gt;$300M Opportunity</td>
<td></td>
</tr>
</tbody>
</table>

*US, EU, JPN prevalence:

~12k-15k
~60k-100k
~8k-10k
~1-2k*
Fintepla® | Dravet Syndrome
Lennox-Gastaut Syndrome
CDKL5 Deficiency Disorder
Additional Rare, Treatment-Resistant Epilepsies
Fintepla® (ZX008, fenfluramine oral solution)

- **FDA and EMA approved in 2020** for the treatment of seizures associated with Dravet syndrome in patients 2 years of age and older
- Launched in U.S. and Germany and cATU authorization in France
- Three positive global pivotal global Phase 3 trials completed
- **Primary endpoint and all key secondary endpoints met with high statistical significance**
- Ongoing OLE has shown robust efficacy results up to three years

**Dravet Syndrome**

- Positive pivotal global Phase 3 trial completed
- Top-line results announced Q1 2020
- **Primary endpoint met with high statistical significance at 0.7 mg/kg dose; several key secondary endpoints met with statistical significance**
  - sNDA submission planned in the U.S. for second half 2021

**Lennox-Gastaut Syndrome**

- Phase 3 efficacy study in CDKL5 deficiency disorder to start second half 2021
- Other treatment-resistant epilepsies e.g., Doose syndrome, sunflower syndrome, etc. through investigator-initiated studies

**CDKL5 Deficiency Disorder, Other Epilepsies**
Dravet Syndrome

A rare and frequently catastrophic epilepsy

Affects 1/15,700 live births in the U.S. (about 200-250 infants/year)

Highly treatment resistant
- 72% of patients take 3 or more antiepileptic drugs
- >80% of patients are inadequately controlled on traditional antiepileptic treatment regimens

Significant morbidity
- High seizure frequency; significant developmental and motor impairments

Increased risk of death
- 6x-higher risk of death, most commonly due to SUDEP (sudden unexpected death in epilepsy) and Status Epilepticus
- 46% of caregivers use pulse-ox at night due to fear of death from SUDEP

Urgent need to reduce or eliminate high seizure burden and associated comorbidities

Dravet Syndrome Phase 3 Trial Efficacy Results

Primary Endpoint Analysis of % Difference From Placebo in Reduction in Mean Monthly Convulsive Seizures

Study 1

- 0.7 mg/kg/day: 70.0% reduction
- 0.2 mg/kg/day: 31.7% reduction

- p-value compared to placebo: p<0.001
- p-value: p=0.043

Study 2

- 0.4 mg/kg/day: 59.5% reduction

- p-value compared to placebo: p<0.001

Source: Fintepla prescribing information

1. All 0.4 mg/kg/day patients were also taking concomitant stiripentol, which increases the exposure of Fintepla.
Additional Study 1 and Study 2 Efficacy Results

**Study 1**

**Proportion of Patients by Category of Seizure Response for FINTEPLA and Placebo in Patients with Dravet Syndrome (Study 1)**

- **Placebo**
- **0.2 mg/kg/day**
- **0.7 mg/kg/day**

**Cumulative 50% or >**
- **0.7 mg/kg** 70.0%
- **0.2 mg/kg** 34.2%
- **Placebo** 7.7%

**Median Longest Interval Between Convulsive Seizures in Patients with Dravet Syndrome (Study 1)**

- **Placebo**
- **0.2 mg/kg/day**
- **0.7 mg/kg/day**

- **<0** days: 7.8
- **≥0 to <25** days: 12.9
- **≥25 to <75** days: 20.5

**p=0.043**

**p<0.001**

**Study 2**

**Proportion of Patients by Category of Seizure Response for FINTEPLA and Placebo in Patients with Dravet Syndrome (Study 2)**

- **Placebo**
- **0.4 mg/kg/day**

**Cumulative 50% or >**
- **0.4 mg/kg** 53.3%
- **Placebo** 4.8%

**Median Longest Interval Between Convulsive Seizures in Patients with Dravet Syndrome (Study 2)**

- **Placebo**
- **0.4 mg/kg/day**

- **<0** days: 0.7
- **≥0 to <25** days: 34.2
- **≥25 to <75** days: 53.3
- **≥75 to 100** days: 7.7

**Cumulative 50% or >**

**p=0.012**

**p-value compared to placebo**

Source: Fintepla prescribing information

All 0.4 mg/kg/day patients were also taking concomitant stiripentol, which increases the exposure of Fintepla.
Dravet Syndrome Study 1503 Open Label Extension Study

Robust seizure reduction sustained in OLE at up to three years with no evidence of waning therapeutic effect

Reduction in Median Mean Convulsive Seizure Frequency (MCSF) Over Time During Fenfluramine Treatment in Open Label Extension (OLE) Study

Median Treatment Duration: 631 days (7-1086)

Median Reduction in MCSF: -83%, P=0.002

The decrease in patient number is primarily due to staggering study entry and not due to patient withdrawal.

Data originally presented at AAN 2021
Safety Highlights in Dravet Syndrome

Fintepla is only available through the Fintepla REMS Program
  • Fintepla is available only through a restricted distribution program called the Fintepla REMS because of the risk of valvular heart disease and pulmonary arterial hypertension.

Boxed Warning: Valvular Heart Disease and Pulmonary Arterial Hypertension
  • There is an association between serotonergic drugs with 5-HT2B receptor agonist activity, including fenfluramine (the active ingredient in Fintepla), and valvular heart disease and pulmonary arterial hypertension.
  • Echocardiogram (ECHO) assessments are required before, during, and after treatment with Fintepla.

Contraindications
  • Fintepla is contraindicated in patients with hypersensitivity to fenfluramine or any of the excipients in Fintepla and with concomitant use of, or with 14 days of, the administration of monoamine oxidase inhibitors because of an increased risk of serotonin syndrome.

No patient has developed valvular heart disease or pulmonary arterial hypertension in controlled or uncontrolled studies of up to 3 years in duration.

*Please see full Prescribing Information for additional Important Safety Information and a complete list of the adverse events reported to date
Fintepla Commercialization

HCP, caregiver awareness; integrated support from enrollment through continued use

Clinical Education

- Clinician and caregiver education about unmet medical needs and Fintepla® clinical data

Zogenix Central Hub

- Integrated support programs and specialty pharmacy distribution

Long-Term Support

- Dedicated case management to ensure proper monitoring and education

U.S. & Europe: Zogenix in-house commercial teams

Japan: Commercial partner Nippon Shinyaku Co. Ltd.
Fintepla U.S. Launch in Dravet Syndrome

HCP Segments

Community HCPs
Treating ~10% Patients

Secondary Epilepsy Centers
Treating ~10% Patients

Top Epilepsy Centers
Treating ~80% Patients

Clinical Trial and EAP Sites

Multi-Pronged Approach under COVID-19

Virtual Call
On Demand Resources
Emails
Speaker Program
Social Media
Digital Display

Traditional Tools used by KAMs

- Reprint Carriers
- Live KAM details
- Websites
- Dosing & Titration Guidelines
- Journal Ads
Zogenix Central

- HCP and Patient – Therapy Decision
- HCP and Patient Enrollment in REMS and Z Central
- Welcome Call – Case Manager
- Reimbursement Assistance
- Therapy Education by Case Manager & Pharmacist
- Z Central follow up with Patient for Refills
- Specialty Pharmacy Ships Drug to Patient
Study 3: Global Phase 3 Trial Including Japan

Primary Endpoint and Secondary Endpoints Met, Consistent with Prior Phase 3 Trials of ZX008 in Dravet Syndrome Patients

Fintepla was generally well-tolerated in this study, with adverse events consistent with those observed in Study 1 and Study 2 and with the known safety profile of fenfluramine.

Primary Endpoint Analysis of % Difference From Placebo in Reduction in Mean Monthly Convulsive Seizures (Study 3)

- Placebo: 0.2 mg/kg/day
- Placebo: 0.7 mg/kg/day

<table>
<thead>
<tr>
<th>Seizure Response</th>
<th>Placebo 0.2 mg/kg/day</th>
<th>Placebo 0.7 mg/kg/day</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>≥50% Reduction</td>
<td>64.8%</td>
<td>49.9%</td>
<td>p&lt;0.0001</td>
</tr>
<tr>
<td>≥75% Reduction</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

p-value compared to placebo

Study 3 was a multi-national, randomized, double-blind, placebo-controlled, Phase 3 study enrolling 143 children and young adults with Dravet syndrome, whose seizures were not adequately controlled by existing anti-epileptic drugs planned to serve as pivotal study for a planned new drug application (J-NDA) in Japan. Top-line data announced September 10, 2020.
Lennox-Gastaut Syndrome

Rare, highly refractory, and notoriously difficult to treat

- Childhood onset, usually between 2-7 years of age
- Varied disease etiology
- Significant intellectual, behavioral, and motor disabilities
- Higher risk of status epilepticus and sudden death
- Most patients’ seizures remain uncontrolled on current antiepileptic treatment regimens
- Accounts for ~1-4 % of all cases of childhood epilepsy\(^1\)
- 60,000-100,000 living with LGS in the U.S. and Europe combined\(^2\)

Patients require constant care, creating high physical, emotional, and financial burden for caregivers and the entire family

---

2. Zogenix company estimates
Primary endpoint met demonstrating Fintepla, at a dose of 0.7 mg/kg/day, was superior to placebo as adjunctive therapy in the treatment of LGS based on median percent change in monthly frequency of seizures that result in drops (p=0.0012)

Key secondary endpoint met: number of subjects with ≥50% reduction in drop seizures: Fintepla 0.7 mg/kg/day group compared to placebo

p-values are treatment compared with placebo
LGS Study 1601 Additional Efficacy Analyses

Subgroup Analysis by Seizure Types, Median Percentage Seizure Reduction From Baseline

**Generalized Tonic-Clonic (GTC) Seizures**

- 0.7 mg/kg/day FFA (n=38): 45.7%
- 0.2 mg/kg/day FFA (n=38): 58.2%
- Placebo (n=38): -3.7%

P = 0.0007

**Tonic-Atonic Seizures**

- 0.7 mg/kg/day FFA (n=13): 46.7%
- 0.2 mg/kg/day FFA (n=21): 13.7%
- Placebo (n=20): 6.8%

P = 0.0455

Data originally presented at AAN 2021
Study 1601 Safety Results

- Generally well-tolerated with adverse events consistent with known safety profile of fenfluramine
- No cases of valvular heart disease or pulmonary arterial hypertension
- Six patients in the 0.7/mg/kg/d group had an AE that led to a discontinuation compared to four patients in the 0.2/mg/kg/d group and one patient in the placebo group
- One death during the trial caused by SUDEP in a patient in the 0.7/mg/kg/d group assessed to be unrelated to study drug

<table>
<thead>
<tr>
<th></th>
<th>Placebo (n=87)</th>
<th>0.2 mg/kg/d (n=89)</th>
<th>0.7 mg/kg/d (n=87)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of Subjects With at Least One Treatment Emergent Adverse Event (AE)</td>
<td>79.3%</td>
<td>76.4%</td>
<td>89.7%</td>
</tr>
<tr>
<td>Number of Subjects With at Least One Serious Treatment Emergent Adverse Event (SAE)</td>
<td>4.6%</td>
<td>4.5%</td>
<td>11.5%</td>
</tr>
<tr>
<td>Most Common Adverse Events (&gt;10%)</td>
<td>Placebo (n=87)</td>
<td>0.2 mg/kg/d (n=89)</td>
<td>0.7 mg/kg/d (n=87)</td>
</tr>
<tr>
<td>Decreased Appetite</td>
<td>13 (14.9%)</td>
<td>18 (20.2%)</td>
<td>32 (36.8%)</td>
</tr>
<tr>
<td>Diarrhea</td>
<td>4 (4.6%)</td>
<td>10 (11.2%)</td>
<td>11 (12.6%)</td>
</tr>
<tr>
<td>Vomiting</td>
<td>5 (5.7%)</td>
<td>12 (13.5%)</td>
<td>9 (10.3%)</td>
</tr>
<tr>
<td>Fatigue</td>
<td>11 (12.6%)</td>
<td>8 (9.0%)</td>
<td>16 (18.4%)</td>
</tr>
<tr>
<td>Pyrexia</td>
<td>11 (12.6%)</td>
<td>11 (12.4%)</td>
<td>9 (10.3%)</td>
</tr>
<tr>
<td>Seizure</td>
<td>5 (5.7%)</td>
<td>11 (12.4%)</td>
<td>5 (5.7%)</td>
</tr>
<tr>
<td>Somnolence</td>
<td>10 (11.5%)</td>
<td>10 (11.2%)</td>
<td>15 (17.2%)</td>
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</tbody>
</table>
Fintepla Intellectual Property Position

Patents and regulatory exclusivity protection

Global Commercialization Rights
- Exclusive worldwide license
- Full data and patent rights in rare epileptic encephalopathies

Orphan Drug Exclusivity
- Dravet: 7.5-year Orphan & Pediatric Drug exclusivity
- **LGS**: 7-year Orphan Drug exclusivity
- **U.S.**
- **Europe**
- **Japan**
  - Dravet: 12-year Orphan & Pediatric Drug exclusivity
  - **LGS**: 10-year Orphan Drug exclusivity
  - Dravet & LGS: Seeking Orphan Drug exclusivity

Strong Patent Portfolio
- U.S. & International patents with expiries 2033-2039
- 8 Orange Book listed patents in the U.S.
- Coverage for Dravet syndrome and other rare epileptic encephalopathies
- Coverage for proprietary methods of manufacture (API), drug product formulation (pending), and Safety/REMs
MT-1621 | TK2 Deficiency
MT-1621: Thymidine kinase 2 (TK2) deficiency

Rare, genetic, progressive and often fatal mitochondrial DNA depletion syndrome

- Autosomal recessive mutations in TK2 gene
- First patient described in 2001
- Clinically characterized by severe muscle weakness, profoundly impairing movement, breathing, eating/nutrition and other normal functions
- Untreated patients show progressive decline which is often fatal. No spontaneous recovery has been reported
- Age of onset is prognostic for outcome; younger patients have high mortality
- Currently no approved therapies; treatment limited to supportive care
MT-1621: dC/dT Substrate Enhancement Therapy

An investigational deoxynucleoside combination therapy for TK2 deficiency

- TK2 phosphorylates deoxycytidine (dC) and deoxythymidine (dT), fundamental building blocks in production of mitochondrial DNA (mtDNA)
- MT-1621 uses alternative enzyme phosphorylation pathways and residual TK2 activity to potentially restore mitochondrial function
- Deoxynucleoside combination therapy improved nucleotide balance, increased mtDNA copy number, improved cell function, and prolonged life in preclinical models of TK2d
- Given orally, dissolved in solution
MT-1621: RETRO study preliminary data

Statistically significant survival in treated vs. untreated (natural history) groups

Survival Analysis Curves Modeled from Treated and Modified Untreated Groups

<table>
<thead>
<tr>
<th>Survival Probability</th>
<th>Treated</th>
<th>Natural History</th>
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</thead>
<tbody>
<tr>
<td>1.0</td>
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<tr>
<td>0.8</td>
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<td>0.6</td>
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<td>0.2</td>
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<td>0.0</td>
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</tbody>
</table>

Survival analysis comparing treated patients in RETRO study with untreated patients (TK2d Natural History data set) showed statistically significant effect of pyrimidine nucleosides on survival ($p < 0.004$)

**Functional Outcomes**
- MT-1621 treated patients demonstrated improvements in functional domains (motor, respiratory, feeding)
- A number of MT-1621 treated patients re-acquired previously lost motor milestones (e.g., ambulation, discontinuing respiratory support)

**Safety**
- MT-1621 was generally well tolerated
- Most common treatment-related adverse event: mild to moderate diarrhea (63%)
- Four patients experienced Serious Adverse Events related to study drug
- Two patients discontinued due to increased liver enzymes
Key Financial Data

- $505.1mm Cash and Marketable Securities as of December 31, 2020
- $230mm in Convertible Senior Notes with 2.75% annual interest, due 2027
- $9.5mm of Net Product Sales of FINTEPLA for the year 2020; $8.1mm in first full quarter of launch Q4 2020
- 2020 Operating Expenses of $261.4mm(1)
  - R&D of $138.0mm
  - SG&A of $99.6mm
- 55.7mm basic shares outstanding as of February 19, 2021(2)

(1) Includes acquired in-process R&D of $14.6mm and change in fair value of contingent consideration of $8.6mm.
(2) Does not include 5.3mm options outstanding at a weighted average exercise price of $29.11 and 0.4mm issued but unvested restricted stock units as of December 31, 2020 or 9.5mm shares eligible for conversion form outstanding Convertible Senior Notes at $24.28/share.
## Upcoming Milestones

<table>
<thead>
<tr>
<th></th>
<th>Dravet syndrome</th>
<th>Lennox-Gastaut syndrome</th>
<th>CDKL5 Deficiency Disorder (CDD)</th>
<th>TK2 Deficiency</th>
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<tbody>
<tr>
<td><strong>Q1 2021</strong></td>
<td>Commercial launch in Europe (Germany)</td>
<td></td>
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<tr>
<td><strong>Q2 2021</strong></td>
<td>Commercial launches in additional EU countries</td>
<td>sNDA submission in U.S.</td>
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<td></td>
<td>J-NDA submission in Japan</td>
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<td>MAA submission in Europe</td>
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<td></td>
<td>Study 1601 Japan top-line results</td>
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<td></td>
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<td>First patient enrolled in global Phase 3 study</td>
<td>All clinical, non-clinical and CMC data available for NDA and MAA submissions</td>
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<tr>
<td><strong>Q4 2021</strong></td>
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<td>Study 1601 Japan top-line results</td>
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<td><strong>2022</strong></td>
<td>Marketing approval in Japan</td>
<td>Marketing approval U.S.</td>
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<td>NDA submission in U.S.</td>
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<td></td>
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<td>Marketing approval Europe</td>
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<td>MAA submission in Europe</td>
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Together we can bring hope and support to patients and families impacted by rare diseases.