Corporate Presentation March 4, 2020



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Exicure is developing nucleic acid therapeutics based on our proprietary Spherical Nucleic Acid (SNA) technology

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• GROWING DEVELOPMENT PIPELINE

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- > Partnership with Allergan for hair loss disorders
- > Partnership with Dermelix for Netherton syndrome
- > Expanding preclinical activities in neurology and ophthalmology

• BETTER UPTAKE, GREATER STABILITY

- > 3-D architecture drives better uptake and greater stability
- > Preclinically demonstrated local delivery in CNS, eye, GI tract, liver, lung, skin
- > Applicable for antisense, siRNA, TLR9 modulators, splice-switching approaches

ROBUST IP & FINANCIAL POSITION

- > 85+ patents issued or allowed; 125+ pending patent applications
- \$110.8 MM reported cash at 12/31/19



Spherical Nucleic Acids: a proprietary technology platform EX

SNAs are dense, radial arrangements of synthetic nucleic acids on a nanoparticle





SNA technology is uniquely differentiated

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• Key Benefits: High Cell Uptake; Extra-hepatic Delivery; Enhanced Stability

- > SNAs have high cell uptake without encapsulation or complexation in vitro¹
- > Extra-hepatic delivery shown for SNAs in humans² and mice (in vivo)³
- > Extended therapeutic half-life of SNAs *in vitro* = enhanced stability inside cells⁴

SNA technology enables localized delivery

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Traditional oligo therapies face substantial delivery challenges...



Linear oligos delivered systematically are rapidly cleared into the liver and often chemically modified for stability

SNA technology allows nucleic acids to be delivered to local sites throughout the body, expanding the potential application of nucleic acid therapeutics

SNAs are delivered locally or topically

to target tissue



Development pipeline

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¹ In combination with checkpoint inhibitors

² On October 14, 2019, the shareholders of Allergan plc voted to approve the acquisition of Allergan by AbbVie Inc., which is expected to close in the first quarter of 2020 and is subject to customary regulatory approvals and other customary closing conditions.

NEUROLOGY

Friedreich's Ataxia: A progressive neurodegenerative disorder that begins in childhood

- Rare, life-shortening, debilitating autosomal recessive neuromuscular disorder
- Estimated 5,000 patients in the US and 15,000 patients worldwide
- Currently no approved therapies
- Driven by expansion of GAA triplet in the first intron of frataxin (FXN) gene
 - > Expanded repeat forms intramolecular triple-helix, which impairs transcription and reduces levels of frataxin
 - > After childhood diagnosis, patients become wheelchair-bound in ~10 years and survive into mid-30s
- Exicure strategy: use genetically targeted SNA therapy to remove FXN transcription blockage and increase FXN levels





Extensive preclinical data provide proof-of-concept for the SNA platform's potential applicability in neurological disorders



NEUROLOGY

ATAXIAS, BATTEN DISEASE, ALS, HUNTINGTON'S DISEASE

- Nusinersen is a commercially available linear oligo therapeutic to treat spinal muscular atrophy (SMA)
- In a proof-of-concept nonclinical study, the SNA construct of the drug performed better than nusinersen
- Compared to nusinersen, the SNA construct:
 - Doubled the levels of healthy full-length SMN2
 mRNA and protein in SMA patient fibroblasts
 - Prolonged survival by 4x (maximum survival of 115 days vs 28 days for nusinersen-treated mice)
 - Mitigated the toxicity of nusinersen in mouse model
 - Showed higher persistence in CNS and lower clearance through kidney in rat biodistribution model

SNA treated survive 4X longer than nusinersen treated mice



Survival data presented at 2018 Annual Cure SMA Conference







Significant unmet medical need in rare neurological disorders exiCUre

- Diseases with genetically validated targets known, but few disease modifying therapies exist
- Not readily addressable by traditional therapeutic modalities
- Significant unmet medical need in rare neurological disorders:
 - Spinocerebellar ataxia, Friedreich's ataxia, Batten disease, Ataxia telangiectasia, Angelman syndrome, Huntington's, ALS



EXICURE OPPORTUNITY

Higher potency and longer persistence in animal models suggest potential advantages for frequency of dosing and therapeutic activity in neurological disorders

IMMUNO-ONCOLOGY

AST-008 in Phase 1b/2 trial in combo with Pembrolizumab exicure

• Phase 1b dose-finding stage: Open label, intratumoral administration of AST-008

- Enrollment completion expected in Q1-2020
- Prioritized cancers: Merkel cell carcinoma, cutaneous squamous cell carcinoma, melanoma, head & neck squamous cell carcinoma
- > Checkpoint inhibitor naïve or refractory patients
- Identifying recommended Phase 2 dose, and evaluating safety, PK, PD and efficacy
- Phase 2 expansion stage in Q2-2020
 - Planning to enroll Merkel cell carcinoma patients who have failed anti-PD-(L)1 therapy
 - > Two-stage Phase 2 enrolling up to 29 patients
 - > Safety, PK, PD, and efficacy read outs
 - Considering adding additional cohorts to the trial in other cancers
 - > Up to 15 total sites expected in the US



7 clinical sites open



Preliminary phase 1b/2 data suggest encouraging anti-cancer activity in Merkel cell carcinoma

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• 14 Patients enrolled and treated with AST-008

- Five melanoma patients, four Merkel cell carcinoma ("MCC") patients, two cutaneous squamous cell carcinoma patients, two head and neck squamous cell carcinoma patients, and one mucosal melanoma patient
- > No treatment-related serious adverse events or dose-limiting toxicities have been observed
- Fifth and final dose escalation cohort of phase 1b is open and enrolling

• Available data from the study show:

- > AST-008 administration, alone or in combination with pembrolizumab, produced cytokine and chemokine expression and immune cell activation in patient blood indicative of desired immune activation.
- > Of the 4 MCC patients, one patient, who had previously progressed on anti-PD-1 antibody therapy, has confirmed stable disease with decreased target lesion diameters for a period in excess of 12 weeks, while a second MCC patient experienced a target lesion complete response and a confirmed overall partial response longer than 24 weeks.
- > Nine patients had progressive disease, two patients have not yet been evaluated and one is not evaluable.

PARTNERSHIPS

SNA technology has broad partnership potential



Exicure + Allergan: a hair loss disorders collaboration

- November 2019, initiated a partnership with Allergan to discover and develop two SNA-based treatments for hair loss disorders
 - Exicure responsible for discovery and other costs prior to option exercise by Allergan
 - > Allergan responsible for all costs after option exercise
- Up to \$725MM in development, regulatory and sales milestones
 - > \$25MM upfront payment
 - \$10MM payment to option a program or request Exicure to perform IND-enabling studies
 - Additional \$15MM option payment if Exicure performs INDenabling studies
 - Development and regulatory milestones of up to \$97.5 million per program
 - > Commercial milestones of up to \$265 million per program
 - > Tiered royalties of mid single-digit to mid-teen percentage



- In February 2019, initiated a partnership with Dermelix targeting Netherton syndrome and up to 5 additional rare skin indications
 - Dermelix responsible for clinical development
- Up to \$166M in development, regulatory and sales milestones per indication
 - > \$1M upfront
 - > Additional \$1M for each option exercised (up to 5)
 - > All development funded by Dermelix
 - > Low double-digit royalties on annual net sales



OPHTHALMOLOGY

• We believe the eye is an attractive organ for locally-applied SNAs

- > Small & immune-privileged (<0.1% total body weight)</p>
- > Established and non-invasive clinical assessments
- > Contralateral control eye for effective trial design

• SNAs may possess potential advantages over gene therapy in the eye

- > Intravitreal injections are safer and easier than subretinal injections
- > Tunable and reversible control of target expression
- > Ability to treat toxic gain-of-function diseases and target large genes

• Approximately 250 rare diseases with known genetic targets

- > CLN3 for Batten disease
- > BEST1 for vitelliform macular dystrophy
- > USH2A for Usher syndrome type 2A

Preclinical data provide rationale for expansion into ophthalmological genetic disorders

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OPTHTHALMOLOGY *undisclosed targets*

undisclosed indication

 SNA technology has shown an encouraging biodistribution profile in preclinical ophthalmology studies:

- Distributes to both posterior (retinal) and anterior (cornea) ocular structures
- Exhibits higher distribution and persists longer compared to linear oligonucleotide
- Does not cause inflammation in the eye



- Intravitreal injection of fluorescent-labeled SNA or linear oligo (in rats)
- Tissue harvested 3 hours following intravitreal injection
- · Arrows indicate ganglion cell layer in the retinal surface

BUSINESS





Exclusive, WW license to make, use and sell SNAs for therapeutic applications



IP portfolio includes 85+ issued or allowed patents and 125+ pending applications across multiple nucleic acid modalities



Multiple layers of protection exist extending platform coverage beyond 2030



Management Team

exicure



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2019 Achievements

- > Friedreich's ataxia established as the first neurological indication
- > AST-008 Phase 1b preliminary results in patients with solid tumor
- > Allergan partnership in hair loss disorders; \$25 MM upfront payment
- > Raised approximately \$90.8 MM in gross proceeds from the sale of common stock
- > Up-listed to the NASDAQ Global Market
- > Expanded board of directors and scientific advisory board

2020 Milestones

- > Q2: Expect to launch AST-008 Phase 2 trial in Merkel cell carcinoma
- > Q2: Expect to launch AST-008 Phase 2 trial in cutaneous squamous cell carcinoma
- > Late 2020: Expect to launch IND enabling studies for XCUR-FXN in Friedreich's ataxia
- > Advance Allergan collaboration in hair loss disorders
- > Advance Dermelix collaboration in Netherton syndrome
- > Continue active business development initiatives

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