

Novartis AG
Investor Relations



Novartis Enters into Agreement to Acquire AveXis

Investor Presentation | April 9, 2018



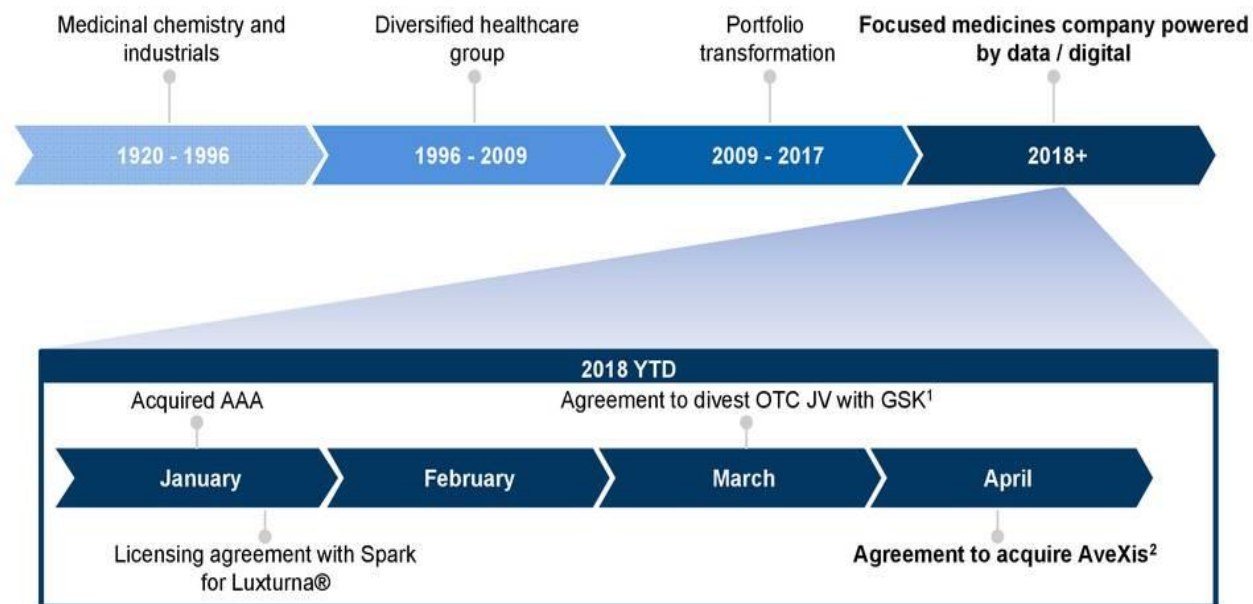
Disclaimer

This presentation contains forward-looking statements that can be identified by terminology such as "potential," "expected," "will," "planned," "pipeline," "outlook," or similar expressions, or by express or implied discussions regarding the potential outcome of the tender offer for the shares of AveXis Inc. to be commenced by Novartis, and the potential impact on Novartis of the proposed acquisition, including express or implied discussions regarding potential future sales or earnings of Novartis, and any potential strategic benefits, synergies or opportunities expected as a result of the proposed acquisition; and regarding potential marketing approvals, new indications or labeling for the potential, investigational or approved products described in this press release, or regarding potential future revenues from such products. You should not place undue reliance on these statements. Such forward looking statements are based on our current beliefs and expectations regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward looking statements. There can be no guarantee that the proposed tender offer or the acquisition described in this press release will be completed, or that it will be completed as currently proposed, or at any particular time. Neither can there be any guarantee that Novartis or any potential products which would be obtained with AveXis will achieve any particular future financial results, or that Novartis will be able to realize any of potential strategic benefits, synergies or opportunities as a result of the proposed acquisition. Nor can there be any guarantee that the potential, investigational or approved products described in this press release will be submitted or approved for sale in any market, or at any particular time. Neither can there be any guarantee that such products will be commercially successful in the future. In particular, our expectations could be affected by, among other things: regulatory actions or delays or government regulation generally, including potential regulatory actions or delays relating to the completion of the potential acquisition described in this release, as well as potential regulatory actions or delays with respect to the development of the products described in this release; the potential that the strategic benefits, synergies or opportunities expected from the proposed acquisition may not be realized or may take longer to realize than expected; the successful integration of AveXis into the Novartis Group subsequent to the closing of the transaction and the timing of such integration; potential adverse reactions to the proposed transaction by customers, suppliers or strategic partners; dependence on key AveXis personnel and customers; the uncertainties inherent in the research and development of new healthcare products, including clinical trial results and additional analysis of existing clinical data; our ability to obtain or maintain proprietary intellectual property protection; safety, quality or manufacturing issues; global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures; the particular prescribing preferences of physicians and patients; uncertainties regarding actual or potential legal proceedings, including, among others, potential legal proceedings with respect to the proposed acquisition; and other risks and factors referred to in Novartis AG's current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this presentation as of this date and does not undertake any obligation to update any forward-looking statements as a result of new information, future events or otherwise.

Important Information and Where To Find It

In connection with the proposed acquisition, Novartis and an indirect wholly-owned subsidiary of Novartis ("Purchaser") will commence a tender offer for the outstanding shares of common stock of AveXis, Inc. (the "Company"). This communication is for informational purposes only and is neither an offer to purchase nor a solicitation of an offer to sell securities. The tender offer for the shares of common stock of the Company has not commenced. At the time the tender offer is commenced, Novartis and Purchaser will file, or will cause to be filed, a Schedule TO Tender Offer Statement with the U.S. Securities and Exchange Commission (the "SEC") and the Company will file a Schedule 14D-9 Solicitation/Recommendation Statement with the SEC, in each case with respect to the tender offer. The Schedule TO Tender Offer Statement (including an offer to purchase, a related letter of transmittal and other offer documents) and the Schedule 14D-9 Solicitation/Recommendation Statement will contain important information that should be read carefully before any decision is made with respect to the tender offer. Those materials and all other documents filed by, or caused to be filed by, Novartis and Purchaser and the Company with the SEC will be available at no charge on the SEC's website at www.sec.gov. The Schedule TO Tender Offer Statement and related materials also may be obtained for free under the "Investors-Financial Data" section of Novartis' website at <https://www.novartis.com/investors/financial-data/sec-filings>. The Schedule 14D-9 Solicitation/Recommendation Statement and such other documents also may be obtained for free from the Company under the "Investor + Media" section of the Company's website at <http://investors.avexis.com/phoenix.zhtml?c=254285&p=irol-IRHome>.

Recent deals in line with strategy to focus on core business



¹ Subject to approval by GSK's shareholders and no governmental orders restraining or prohibiting the transaction.

² Subject to customary closing conditions. Until closing, AveXis will continue to operate as a separate and independent company.

Five key priorities to shape our future

Agreement to acquire AveXis¹ is in line with the Novartis vision to deliver transformative innovation in areas of high unmet medical need

Operational Execution



Ensure launch excellence, high levels of productivity, and margin improvement

Breakthrough Innovation



Focus on high-end, transformative innovation

Data / Digital Leadership



Pivot to become a data-centric, digitally powered company

Trust & Reputation



Rebuild trust with society




Culture Transformation



Shift to a more empowered, inspired, unbureaucratic organization

¹ Subject to customary closing conditions. Until closing, AveXis will continue to operate as a separate and independent company.

Strategic Rationale: Novartis expands position as a leader in gene therapy and Neuroscience

	Potentially Transformative Treatment for SMA (Spinal Muscular Atrophy)	<ul style="list-style-type: none">▪ Potential transformational gene replacement therapy; if approved, it could save lives & improve motor function in an area of significant unmet need
	Provides Capabilities in Gene Therapy	<ul style="list-style-type: none">▪ Provide R&D capabilities in Adeno-Associated Virus 9 (AAV9) platform, which has broad applicability including for Novartis' internal portfolio▪ Obtain access to newly built manufacturing capabilities in gene therapy
	Supports Neuroscience Strategy	<ul style="list-style-type: none">▪ Potential for broader utility with aim to expand platform to Rett Syndrome (RTT) & ALS-SOD1¹

¹ ALS-SOD1 is a genetic form of amyotrophic lateral sclerosis (ALS) caused by mutations in the superoxide dismutase 1 (SOD1) gene

Overview of AveXis

Company History & Overview

- Clinical-stage gene therapy company focusing on rare and life-threatening diseases, notably SMA
- Incorporated in 2010; IPO on Nasdaq in February 2016
- Headquartered in Bannockburn, Illinois



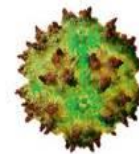
AVXS-101 Background

- Proprietary **gene replacement therapy product candidate for the treatment of SMA**
- **Breakthrough therapy designation** from FDA for treatment of SMA Type 1
- **PRIME** designation from EMA
- **Sakigake** designation from Japan's MHLW
- AVXS-101 would be a **one-time treatment** to restore production of SMN protein and to prevent further degeneration, thereby **increasing motor function and enhancing survival**



Gene Therapy Pipeline / Platform

- Platform is based on **AAV9 technology**
- AAV9 has shown to efficiently cross the blood brain barrier – which makes it an **attractive vehicle for CNS diseases**
- Drug candidates based on AAV9 for **Rett Syndrome & ALS-SOD1** in pre-clinical development
- Gene therapy manufacturing facility in Libertyville, Illinois



Transaction Highlights (expected closing mid 2018)

Consideration

- AveXis shareholders to receive USD 218 per share in cash
- Values AveXis at approximately USD 8.7bn on a fully diluted equity basis

Financing

- The transaction to acquire AveXis is planned to be funded through available cash and short-term borrowing
- Novartis intends to re-deploy the proceeds of the sale of our OTC JV stake to fund the acquisition. This is subject to the timing of closing of the transactions

Financial Benefits

- Expected to contribute to Group sales in 2019 and significant ramp-up in 2020
- Expected to be significantly accretive to Group Core OpInc and Core EPS from 2020; Slight dilution in EPS in 2018 and 2019 (mainly due to R&D)
- Expected IRR well in excess of cost of capital

Other

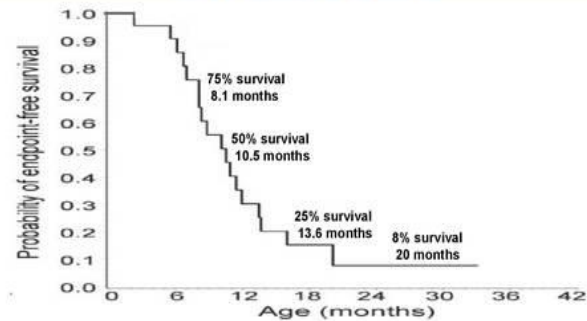
- Transaction unanimously approved by the Boards of Directors of both companies
- Closing expected mid 2018, subject to success of the tender offer and satisfaction of customary closing conditions

Natural history of SMA Type 1

90% of children will not survive 2 years of life or become ventilator dependent

- Apart from high rates of ventilatory and tube-feeding support, SMA Type 1 infants will never be able to sit unassisted
- Prolongation of survival with supportive care does not allow new motor milestones in these infants
- Loss of motor neurons starts very early in life and continues over time; the highest motor milestone achieved is typically seen in the infant's first visit, and is followed by a continued, rapid decline

Endpoint-Free¹ Survival



Motor Milestones²

Normal Development	SMA Type 1 (at best)
Holds head steady (~4 months)	Partial, wobbling
Rolls over (~6 months)	Very rarely, partial
Sits alone, crawls (~8 months)	Never
Cruises, may stand alone (~12 months)	Never

¹ PNCR (Finkel); Survival = no death and no need for ≥16 hr/day ventilation continuously for ≥2 weeks, in the absence of an acute reversible illness (n=23) – 2 SMN2 copies

² De Sanctis R, et al. Neuromuscul Disord. 2016; 26:754-9

SMA – an area of significant unmet need affecting ~23,500 patients worldwide (established markets¹)

	Type 1	Type 2	Type 3	Type 4
SMN2 Copy Number	2	3 ⁴	3 or 4	4 to 8
Onset	Before 6 months	6 – 18 months	Early childhood to early adulthood (juvenile)	Adulthood (20s-30s), usually after 30
Incidence split	~ 60%	~ 27%	~ 13%	Uncommon / limited information available
Prevalence split ²	~ 14%	~ 51%	~ 35%	Uncommon / limited information available
Est. WW Prevalent Population	~ 3,300	~ 12,000	~ 8,200	Uncommon / limited information available
Development Milestones	<ul style="list-style-type: none"> • Will never be able to sit without support • Difficulty breathing / swallowing • Cannot crawl / will never walk 	<ul style="list-style-type: none"> • Will never be able to walk without support • Most will never stand without support 	<ul style="list-style-type: none"> • Stand unassisted and walk independently, but may lose ability to walk over time 	<ul style="list-style-type: none"> • Stand alone and walk but may lose ability to walk in 30s-40s
Survival	Over 90% die or have significant critical event ³ by age 2	32% die before age 25	Normal	Normal

Source: AveXis company presentation (Dec 13, 2017)

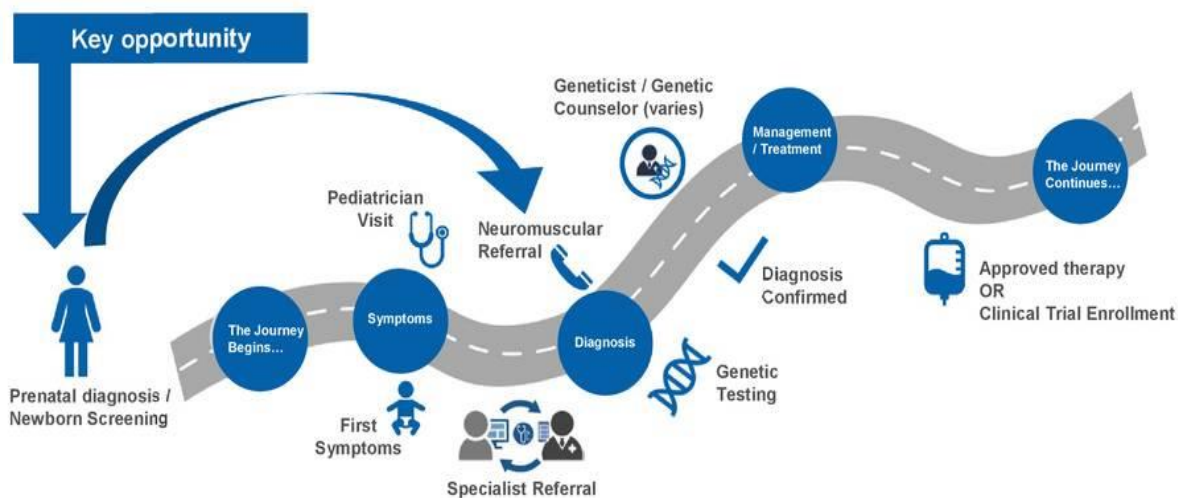
¹ Estimate US, Japan, EU15, Australia, Canada, Turkey

² Spinal Muscular Atrophy: Introduction to SMA families: SMA Foundation

³ Event = Death or ≥ 16 hr/day ventilation continuously for ≥ 2 weeks, in the absence of acute reversible illness

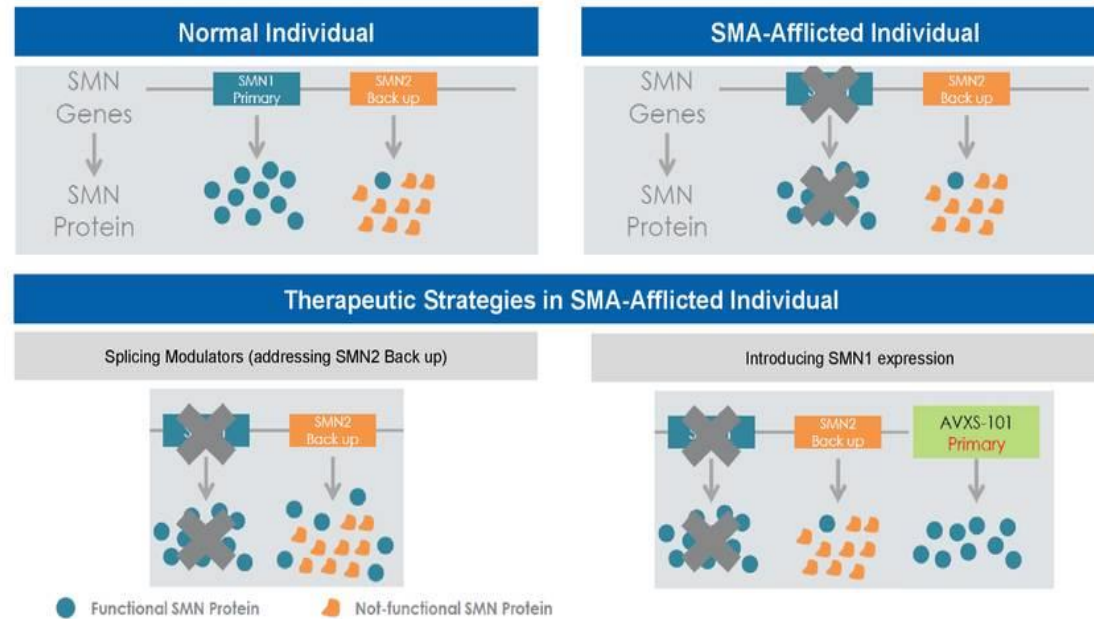
⁴ 100% have 3 copies (PNCr)

Expected US newborn screening could transform SMA care



- 'Time = neurons': All available treatments work better when started earlier; lost function unlikely to be regained
- Clear progress in US Federal and State newborn screening for SMA, EU and other countries are also evaluating
- Expected outcome is that the Type 1/2/3 categorization based on motor milestones will be replaced by SMN2 gene copy assessment and genetic diagnosis of expected early-onset or late-onset SMA

Mechanism of disease and therapeutic strategies



Source: AveXis company presentation (Dec 13, 2017)

AVXS-101: Potential first gene replacement therapy for SMA

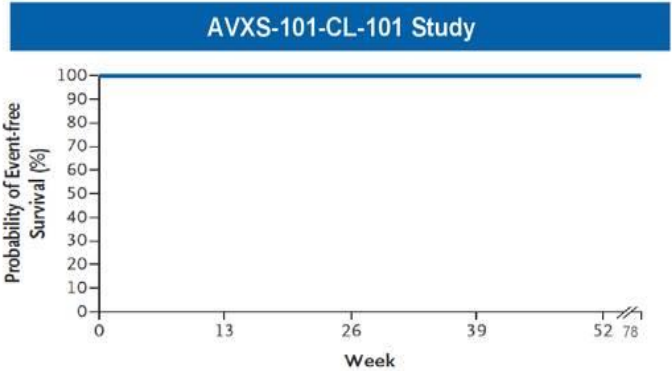
Gene therapy is the right approach for SMA: Monogenic mutation that drives the pathology



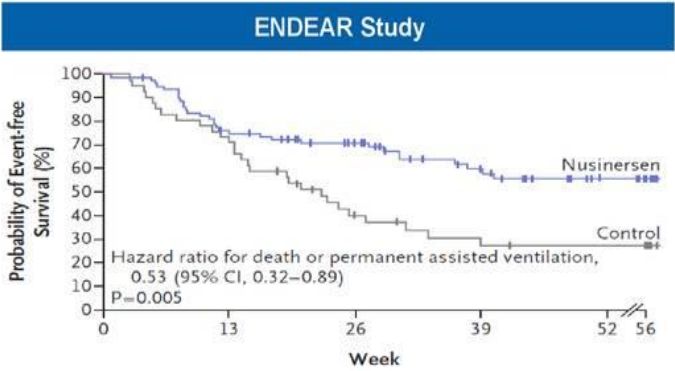
scAAV ITR	Continuous Promoter	Human SMN Transgene	scAAV ITR
KEY COMPONENTS		PURPOSE	
Recombinant AAV9 Capsid Shell		<ul style="list-style-type: none">Ability to deliver across the blood brain barrier and into the spinal cord (can be administered systemically)Non-replicating virus does not modify the existing DNA of the patient	
scAAV ITR (Self-complementary DNA technology)		<ul style="list-style-type: none">Enables rapid onset of effect which is key in a quickly deteriorating population	
Continuous Promoter		<ul style="list-style-type: none">Activates the transgene to allow for continuous and sustained SMN expression	
Human SMN Transgene		<ul style="list-style-type: none">Full copy of a stable functioning SMN gene that is introduced into the cell's nucleus	

Rendering adapted from Dimattia et al. Structural Insight into the Unique Properties of Adeno-Associated Virus Serotype 9. J. Virol. June 2012.

Event-free survival in SMA Type 1



Mendell JR, et al, N Engl J Med 2017; 377:1713-1722



Finkel RS, et al, N Engl J Med 2017; 377:1723-32

AVXS-101-CL-101 Study		ENDEAR Study
Diagnosis	SMA Type 1, Symptomatic, 2 SMN2 Copies	SMA Type 1, Symptomatic, 2 SMN2 Copies
Drug Studied	AVXS-101	Nusinersen
Mean age (Month)	3.4 (0.9 – 7.9) - High dose 6.3 (5.9 – 7.2) - Low dose	5.36 (1.7 – 7.9)
Permanent ventilatory assistance definition	≥16 hrs of respiratory assistance/ day for ≥ 14 consecutive days in absence of an acute, reversible illness or a peri-operative state	Tracheostomy or ventilatory support for ≥ 16 hrs/ day for more > 21 consecutive days in the absence of acute reversible event

Studies (AVXS-101-CL-101 and ENDEAR) differ in patients' age and definition of ventilatory support

CL-101 Clinical Trial: Motor milestone achievement assessed and adjudicated by independent external reviewer (Aug 7, 2017)

Two children crawl, pull to a stand, and stand and walk independently									
Cohort 2 2.00E+14 VG/KG	Age at GT (Months)	Motor Milestone Achievement							
		Brings hand to mouth	Head Control	Partial Roll ¹	Roll ²	Sitting with Assistance	Sitting Unassisted		
							≥5 Seconds ³	≥10 Seconds ⁴	≥30 Seconds ⁵
E.04	6	✓	✓	✓	✓	✓	✓		
E.05	4	✓	✓	✓	✓	✓	✓	✓	✓
E.06	2	✓	✓	✓	✓	✓	✓	✓	✓
E.07	4	✓	✓	✓	✓	✓	✓	✓	
E.08	8	✓							
E.09	5	✓	✓	✓	✓	✓	✓	✓	✓
E.10	1	✓	✓	✓	✓	✓	✓	✓	✓
E.11	2	✓	✓	✓	✓	✓	✓	✓	✓
E.12	3	✓	✓	✓	✓	✓	✓	✓	✓
E.13	1	✓	✓			✓	✓	✓	✓
E.14	4	✓	✓	✓	✓	✓	✓	✓	✓
E.15	2	✓	✓	✓		✓	✓	✓	✓

Source: AveXis press release (Nov 1, 2017)

¹ Bayley Scales of Infant and Toddler Development, item #20, rolls a minimum 180° from back in only one direction

² Bayley Scales of Infant and Toddler Development, item #20, rolls a minimum 180° from back to both left and right

³ Sitting unassisted for ≥5 seconds is in accordance with the criteria of item 22 in the Bayley Scales of Infant and Toddler Development – gross motor subtest and surpasses the three second count used as a basis for sitting (test item 1) in the Hammersmith Functional Motor Scale – Expanded for SMA (HFMSE)

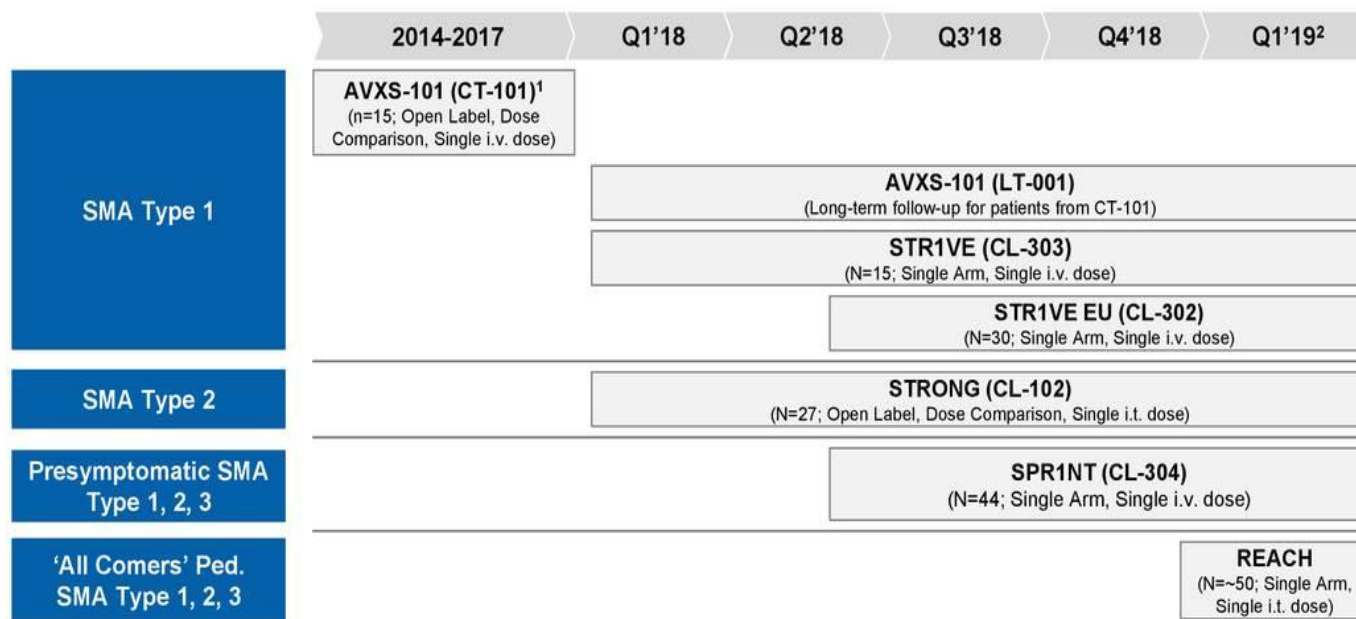
⁴ Sitting unassisted for ≥10 seconds is in accordance with the criteria in the World Health Organization – MultiCentre Growth Reference Study

⁵ Sitting unassisted for ≥30 seconds defines functional independent sitting and is in accordance with the criteria of item 26 in the Bayley Scales of Infant and Toddler Development – gross motor subtest

Additional long-term data to be presented at AAN on April 25



AVXS-101 SMA expanded clinical development plan



¹ AVXS-101-CT-101 study published in N Engl J Med 2017; 377:1713-1722

² All studies continue beyond Q1 2019; i.v. - Intravenous; i.t. - Intrathecal

AVXS-101 Regulatory Update



- Breakthrough therapy granted (July 2016)
- Received approval to initiate STR1VE and STRONG studies
- Pre-BLA meeting planned for Q2 2018
- BLA submission planned for H2 2018



- PRIME designation granted (Jan 2017)
- Scientific advise received (STR1VE EU study underway)
- File submission planned for H2 2019



- Sakigake designation granted (March 2018)
- Pre-submission discussions planned Q3 2018

AveXis has made significant progress in advancing its manufacturing capabilities ahead of commercialization

- Redesigned the Phase 1 clinical process into a manufacturing process capable of meeting patient demand post approval
- Completed build-out of AveXis manufacturing site in Libertyville, IL and planning additional facilities for capacity build
- Manufactured clinical supply, executed process validation and currently building commercial launch inventory
- Preparing for BLA submission and pre-license inspection



Source: AveXis, Inc.

AveXis offers an attractive gene therapy platform with potential beyond SMA

- AAV9 is considered an optimal gene delivery platform for the CNS: infects cells in the CNS when administered i.v. and i.t., demonstrated good safety and efficacy results in patients
- AveXis has research expertise in developing AAV based gene vectors and delivering them to patients
- IP for AAV9 delivery of genes for SMA, Rett Syndrome and inherited ALS-SOD1
- Suitable for a broad variety of other monogenic diseases (e.g. Fragile X, PMDS, Dravet Syndrome etc.) as well as for delivering antibodies and other biologics into the CNS for more common diseases
- Manufacturing capacity and expertise with potential to accelerate future gene therapy programs and launches

Selected assets	Indication	Status
AVXS-101 (AAV9)	SMA	Pivotal studies
CGF166 (AAV5)	Hearing loss	Phase 1b
CPK850 (AAV8)	Retinitis pigmentosa	Phase 1b
AVXS-201 RTT	Rett Syndrome	Preclinical
AVXS-301 SOD1	Inherited ALS-SOD1	Preclinical
Homology Medicines collaboration	Ophthalmology & hematology	Preclinical

AveXis plans to advance two new gene therapy products into the clinic in late 2018 / early 2019

AVXS-201 for Rett Syndrome		AVXS-301 for Genetic ALS with SOD1 Mutation	
Rett Syndrome	<ul style="list-style-type: none"> Rare, neuro-developmental genetic disorder characterized by slowed growth, loss of normal motor movement and coordination; and loss of communication skills Caused by an X-linked dominant mutation in the methyl CpG binding protein 2 (MECP2) gene Median age at diagnosis is 2.7 years 	Genetic ALS with SOD1 Mutation	<ul style="list-style-type: none"> Rare, neurodegenerative genetic disorder that affects nerve cells in the brain and the spinal cord and leads to progressive degeneration of motor neurons Caused by mutations by the gene that produces the copper zinc superoxide dismutase 1 (SOD1 enzyme)
AVXS-201 Mechanism of Action	<ul style="list-style-type: none"> Composed of a recombinant AAV9 capsid shell, with a human transgene and a continuous promoter specifically designed for optimal MECP2 expression 	AVXS-301 Mechanism of Action	<ul style="list-style-type: none"> Composed of a recombinant AAV9 capsid shell, with a human transgene and a continuous promoter specifically designed for optimal MECP2 expression Polymerase III promoter to drive expression of shRNA to suppress SOD1
AveXis Next Steps	<ul style="list-style-type: none"> Complete remaining IND-enabling preclinical work KOL meetings for both AVXS-201 and AVXS-301 have occurred and clinical plans are forthcoming Submit IND applications for both indications in late 2018 / early 2019 		

Source: AveXis company presentation (Feb 27, 2018)

Expected next steps

AveXis Development and Regulatory Status

- Long-term data for CL-101 at AAN on April 25, 2018
 - US: Pre-BLA meeting in Q2 2018; file submission in H2 2018
 - EU: File submission in H2 2019
 - Ongoing recruitment for STR1VE and STRONG
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Novartis Transaction

- Novartis to commence tender offer for the outstanding shares of common stock of AveXis, Inc.
- Closing expected mid 2018, subject to success of the tender offer and satisfaction of customary closing conditions

Note: The acquisition of AveXis is subject to customary closing conditions. Until closing, AveXis will continue to operate as a separate and independent company.