

Zolgensma[®] & Piqray[®] FDA approvals

Investor Relations

Vas Narasimhan, CEO, Novartis

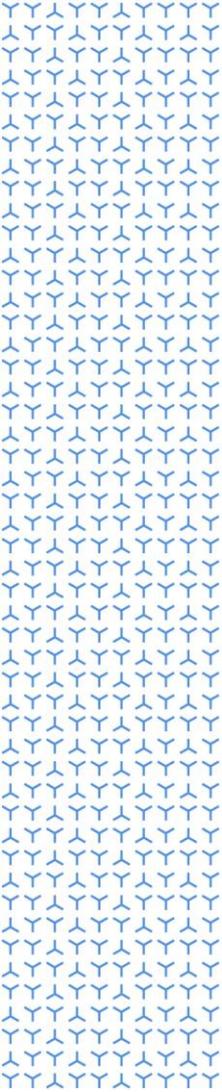
Paul Hudson, CEO, Novartis Pharmaceuticals

Dave Lennon, President, AveXis

May 27, 2019

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Zolgensma[®]

Zolgensma® the first-ever gene therapy for spinal muscular atrophy (SMA)



- Indicated for the treatment of **pediatric patients less than 2 years of age with SMA**
- Approved by FDA ahead of PDUFA date under Priority Review
- Rare Pediatric Priority Review voucher granted
- Launching immediately with product available to ship within the next 2 weeks



Please read full Prescribing Information for Zolgensma®, including Boxed Warning for Acute Serious Liver Injury.

Zolgensma[®] approved by FDA for a broad range of pediatric patients with SMA

Indication statement¹

Zolgensma[®] (onasemnogene abeparvovec-xioi) is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1 (SMN1)* gene

Target patient population

- <2 years of age
- Genetic diagnosis of SMA (regardless of *SMN2* copy number)
- Symptomatic or pre-symptomatic

1. US Prescribing Information.

Zolgensma[®] delivered event-free survival, rapid improvement in motor function, and transformative and sustained milestone achievements

	Event-free survival	Motor function (CHOP-INTEND)	Milestone achievement
Natural history	<p>25% at 13.6 months</p> <p>8% at 20 months¹</p>	<p>Always decline in score</p> <p>Almost none reach a CHOP-INTEND score of ≥ 40 after 6 months of age⁴</p>	<p>Never achieve the ability to sit without support¹</p>
STR1VE	<p>87% at 13.6 months^{2,3}</p>	<p>+6.9 points from baseline one month post-treatment⁵</p> <p>95% achieved a score ≥ 40 points³</p>	<p>50% sitting without support ≥ 30 seconds at a mean of 8 months post-treatment (study ongoing)³</p>
START Therapeutic-dose Cohort	<p>100% at 24 months³</p>	<p>+9.8 points from baseline one month post-treatment³</p> <p>92% achieved a score ≥ 40 points³</p>	<p>75% sitting ≥ 30 seconds⁴</p> <p>In long-term follow-up, no previously achieved motor milestone has been lost (mean 3.7 years post-treatment)³</p>

1. Finkel RS, et al. *Neurology* 2014;83(9):810–817. 2. 13 of 15 patients (87%) who had reached 13.6 months of age or discontinued the study prior to 13.6 months were surviving without permanent ventilation. 3. Presented at the 71st American Academy of Neurology, Philadelphia, PA May 4–10, 2019. 4. Lowes L, et al *Pediatric Neurology* 2019; accepted online 13 May 2019. 5. Mendell JR, et al. *N Engl J Med*. 2017;377:1713–22.

Requirements for initiation and monitoring¹

Prior to treatment

Anti-AAV9 antibody titer screen (~2-day turnaround¹)

Assess liver function by clinical examination and by laboratory testing

Baseline platelets and troponin-I

Start steroid course at 1mg/kg/day (1-day prior to treatment)

Treatment day

One-time, one-hour IV infusion

Outpatient administration

Post-treatment

Continue steroids for at least 1 month at 1 mg/kg/day; Assess liver function weekly the 1st month, every other week 2nd, 3rd months

When results of clinical examination and lab testing of liver function are unremarkable, begin steroid taper over next 28 days

Also assess platelets and troponin-I for 3 months

Please read full Prescribing Information for Zolgensma[®], including Boxed Warning for Acute Serious Liver Injury.

¹.Based on experience in Managed Access Program.

Commercial presentation customized for each patient



- Dosed in 0.5kg increments from 2.6-13.5kg based on kits of 2-9 vials¹
- Individual patient kits prepared and shipped from FDA-approved gene therapy manufacturing site in Libertyville, IL
- Shipped on dry ice chain using custom, GPS-tracked, temperature-controlled SavSu shippers using single courier partner

1. US Prescribing Information.

Zolgensma[®] highly cost-effective at USD 2.125m

Available at USD 425K per year for 5 years in installment plan

50%

Below the ultra-rare cost-effectiveness threshold¹

50%

Below the 10-year cost of current chronic SMA drug²

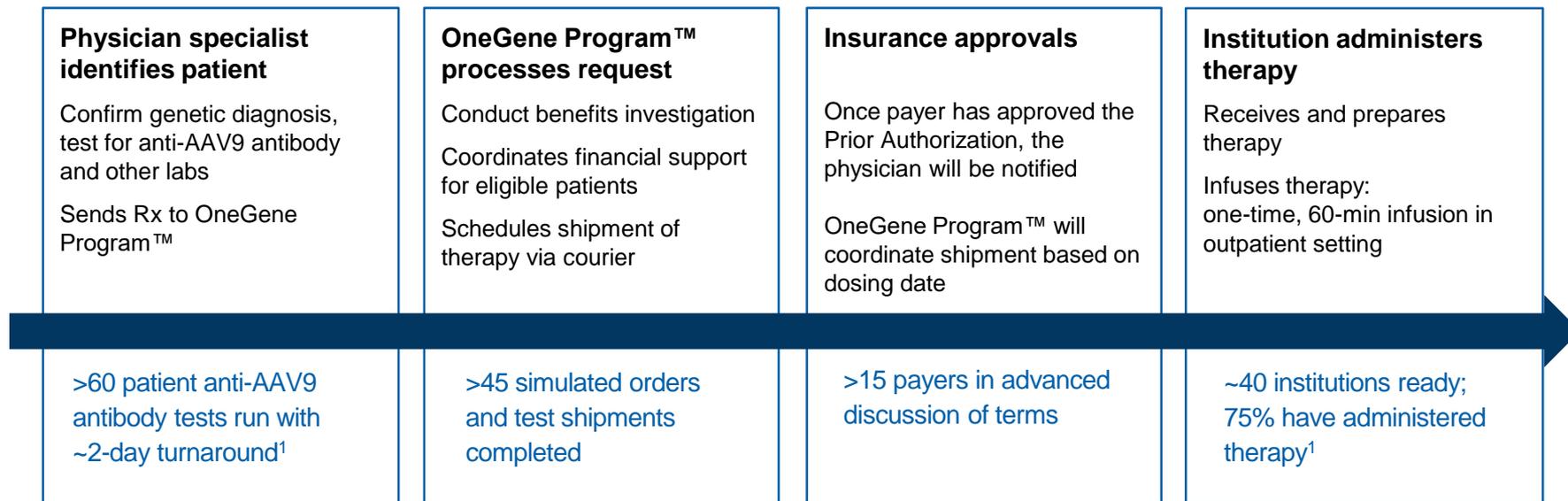
50%

Below the 10-year costs of 5 genetic, pediatric, ultra-orphan treatments^{3*}

Backed by five-year outcomes-based agreements, supported by contracting programs

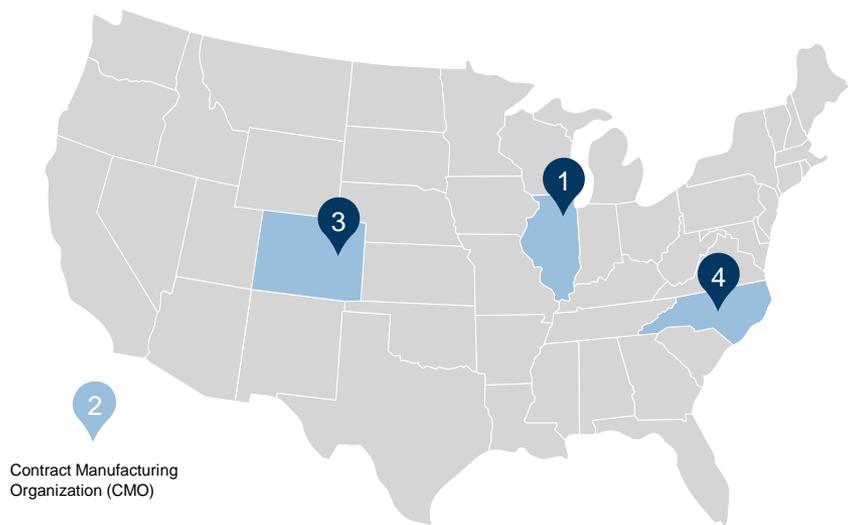
1. ICER. Spinraza[®] and Zolgensma[®] for Spinal Muscular Atrophy: Effectiveness and Value. Feb. 22, 2019 Spinraza[®] is a registered trademark of Biogen. 2. Spinraza[®] WAC. 3. Estim. 10-year cumulative cost based on likely starting age and weight, published dosing guidelines, and per unit WAC cost of therapy by product. Strensiq[®], Soliris[®], Vimizim[®], Cerezyme[®] and HEMLIBRA[®]. Before rebates/discounts. Per package/vial costs from RED BOOK (IBM Watson Health), accessed 2/4/2019. Before rebates/discounts. *Strensiq[®] is a registered trademark of Alexion Pharmaceuticals Inc. Soliris[®] is a registered trademark of Alexion Pharmaceuticals Inc. Vimizim[®] is a registered trademark of BioMarin Pharmaceuticals Inc. Cerezyme[®] is a registered trademark of Genzyme Corporation. HEMLIBRA[®] is a registered trademark of Chugai Pharmaceutical Co., Ltd., Tokyo, Japan.

Strong indicators for marketplace readiness: lab testing, distribution, institutions



1. Based on experience in Managed Access Program. Contracted turnaround is ≤4 days.

Zolgensma[®] production ready for launch and expanding further



Ready for launch

Illinois site approved by FDA for commercial gene therapy manufacturing

Commercial production batches being accumulated since January

Accelerated release protocol agreed with FDA

3 additional sites under development for expansion (AveXis North Carolina & Colorado sites, and third-party CMO)

Potential eligible Zolgensma[®] patients at launch

Prevalent SMA

- Approx. 700 prevalent²
- Previously diagnosed
- Potentially on Spinraza[®]



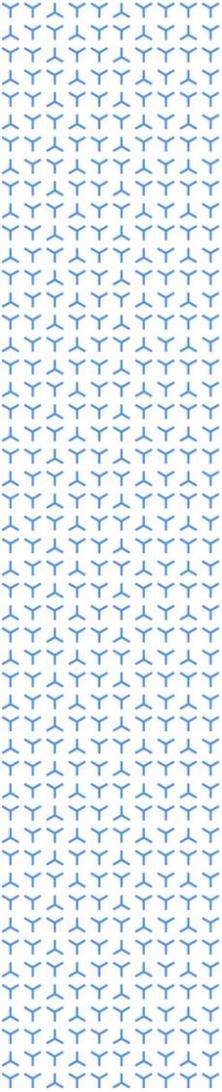
Incident SMA

- Approx. 400 patients¹
- ~30 newly diagnosed per month

1. Assumes 1 in 8,000 SMA births per year. Includes newborn screening diagnosed patients with 2 & 3 SMN2 copy number and symptomatically diagnosed Type 1 & 2 infants. 2. Includes infants diagnosed prior to Zolgensma[®] launch in the last 2 years. Spinraza[®] is a registered trademark of Biogen.

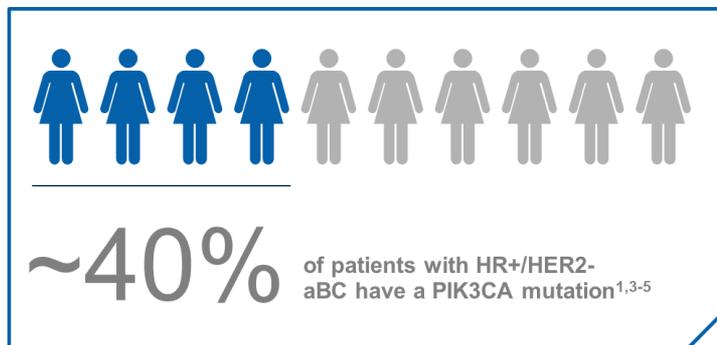
Zolgensma[®] sets new standard as first-and-only, one-time gene therapy for SMA

- ✓ Broad label covering all SMA under the age of 2, including pre-symptomatic
- ✓ Rapid motor function improvements, unprecedented event-free survival and sustained milestone achievements compared to natural history
- ✓ Price significantly discounted vs chronic therapy costs; flexible options in place to support patient access
- ✓ Ready to launch with adequate supply



Piqray[®]

Next pioneering medicine, Piqray[®], the first and only therapy for aBC patients with PIK3CA mutation



- Approved for use in combination with fulvestrant for the treatment of postmenopausal women, and men, with HR+/HER2-, PIK3CA-mutated, advanced or metastatic breast cancer, as detected by an FDA-approved test following progression on or after endocrine-based regimen¹
- ~40% of HR+/HER2- advanced breast cancer patients may face worse disease prognosis due to presence of PIK3CA mutations⁵⁻⁹
- Nearly doubled median PFS in HR+/HER2- aBC patients with a PIK3CA mutation compared to fulvestrant alone¹⁻⁴

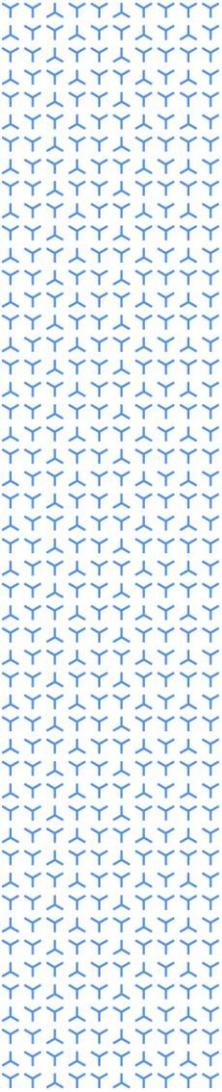
The most common adverse reactions including laboratory abnormalities on the alpelisib plus fulvestrant arm were increased glucose, increased creatinine, diarrhea, rash, decreased lymphocyte count, increased gamma glutamyl transferase, nausea, increased alanine aminotransferase, fatigue, decreased hemoglobin, increased lipase, decreased appetite, stomatitis, vomiting, decreased weight, decreased calcium, decreased glucose, prolonged activated partial thromboplastin time (aPTT), and alopecia. Full safety can be found at <https://www.pharma.us.novartis.com/sites/www.pharma.us.novartis.com/files/piqray.pdf>

1. Piqray (alpelisib) Prescribing Information. East Hanover, New Jersey, USA: Novartis Pharmaceuticals Corporation; May 2019. 2. André F, Ciruelos E, Rubovszky G. Alpelisib for PIK3CA-Mutated, Hormone-Receptor-Positive Advanced Breast Cancer. N Eng J Med 2019. 3. André F, Ciruelos EM, Rubovszky G et al. Alpelisib (ALP) + fulvestrant (FUL) for advanced breast cancer (ABC): Results of the phase III SOLAR-1 trial. Annals of Oncology, Vol 29, Suppl 8, October 2018, Abstract LBA3_PR.4. Juric D, Ciruelos EM, Rubovszky G et al. Alpelisib (ALP) + fulvestrant (FUL) for advanced breast cancer (ABC): Phase 3 SOLAR-1 trial results. Presented at the San Antonio Breast Cancer Symposium (SABCS) (Abstract #GS3-08) on December 6, 2018. 5. Tolaney S, Toi M, Neven P, et al. Presented at: 2019 American Association for Cancer Research (AACR) Annual Meeting; March 29-April 3, 2019; Atlanta, GA. 6. Di Leo A, Johnston S, Seok Lee K, et al. Lancet Oncol. 2018;19(1):87-100. 7. Moynahan ME, Chen D, He W, et al. Br J Cancer. 2017;116(6):726-730002E. 8. The Cancer Genome Atlas Network. Comprehensive molecular portraits of human breast tumours. 2012;490(7418):61-70. 9. Sobhani N, Roviello G, Corona SP et al. The prognostic value of PI3K mutational status in breast cancer: a meta-analysis. J Cell Biochem. 2018;119(6):4287-4292

Launch preparedness of Piqray® in the US



- First new drug application approved under the FDA Oncology Center of Excellence Real-Time Oncology Review pilot program
- Launched with FDA approved companion diagnostic for PIK3CA testing (Qiagen)
- US supply ready for commercial sale
- Entered into agreement with Foundation Medicine to develop plasma and tissue test
- Engaged with payers covering over 80% of the target population in the US
- Continuing to investigate Piqray® in other PIK3CA mutation driven disease states



Q&A