Precision ADVANCE / Cell & Gene Therapy Collective

PRECISION
value & health
shift the trajectory

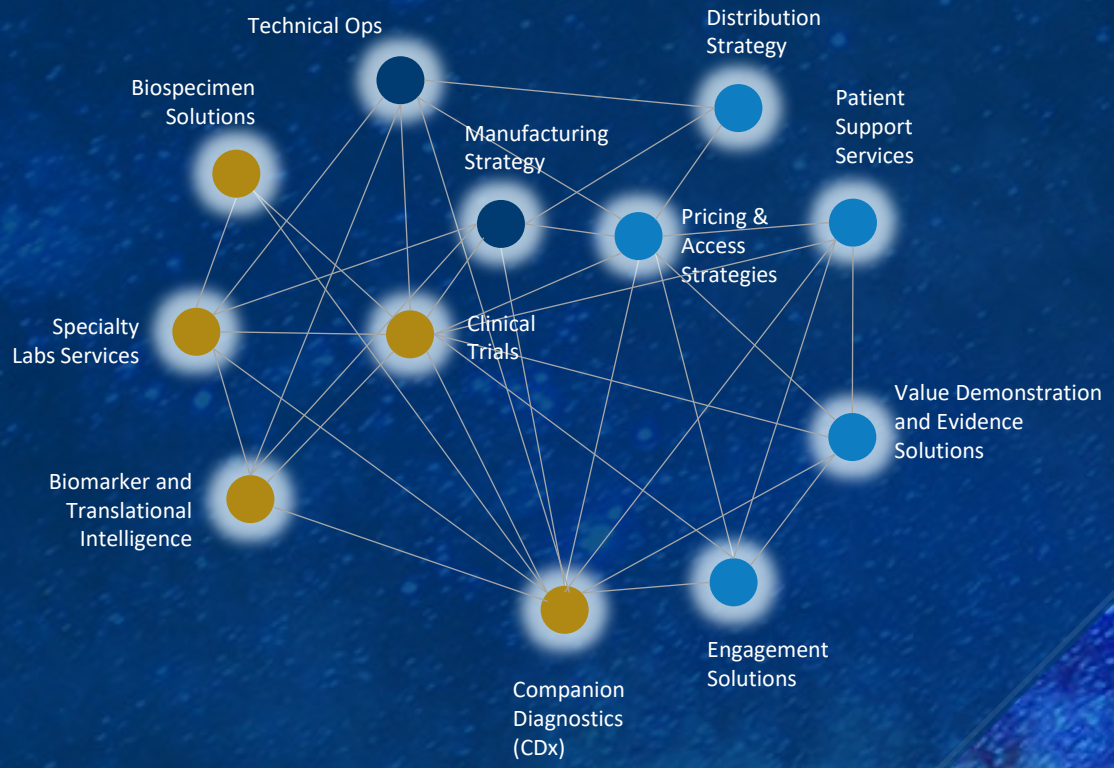
PRECISION ADVANCE the cell & gene therapy collective™

PRECISION value & health

Precision ADVANCE, a collection of interconnected services and complementary teams, uniquely focuses on the complexities of clinical, regulatory, manufacturing, and commercial needs to successfully bring a cell or gene therapy to market

CLINICAL MANUFACTURING COMMERCIAL

ADVANCE was created to capitalize on our collective CGT experience and align with the natural connection in development



Extensive CGT experience across R&D, manufacturing and commercialization

Showcasing our *collective* experience

85+

CGTx Clients

100+

Market Moving Thought Leadership Pieces

90+

Peer-reviewed Publications, Posters, and Panels

40+

Facility Builds and Capital Expansions with $4B+ in Investment Over the Past Four Years

70%+

of Approved Cell and Gene Therapies Support with Commercial Services

Winner

Best Podium at ISPOR [1]

OBN Best Specialty CRO Award [2]

1. ISPOR 2019 - Cost-utility Analysis of Single Dose Gene-replacement Therapy for Spinal Muscular Atrophy Type 1 Compared to Chronic Nusinersen Treatment
2. OBN Awards 2018 and 2019

Precision Is a Global Leader in All Facets of Gene and Cell Therapy Commercialization

Experts in the complexities of the clinical, regulatory, and commercialization challenges in this space

Anticipate and shape next generation healthcare delivery in gene and cell therapy by leading global conversations on all facets of the relevant landscape

- Engagement on nearly all approved gene and cell therapy options in markets across the globe:
  - Technical training on therapies
  - Economic models
  - Design of distribution plans
  - Value stories
  - HCP materials
- Dozens of original research papers, cost-effectiveness analyses, and 50+ posters, podium presentations, and panels
- Ongoing dialogue with the FDA and HTAs regarding gene and cell therapy model conceptualization and use of appropriate costs across the globe

Recent Cell and Gene Therapy Experience

1. Developed cost-effectiveness models for gene therapies treating multiple conditions such as spinal muscular atrophy, XLMTM, MLD, inherited retinal dystrophies and choroideremia

2. Adapted cost-effectiveness model for 19 markets (APAC, EU, Latin America and Canada) and supported successful HTA submissions for SMA

3. Built US payer-facing health economic models for CAR-T therapy indicated for the treatment of adult patients with relapsed or refractory and gene therapy for IRD

4. Created multi-platform initiative to promote the importance of genetic testing for inherited retinal dystrophies to prepare market for new treatments

5. Created initial payer value propositions for upcoming lenti virus gene therapy platform launching in two indications for rare diseases

6. Developed the distribution strategy for one of first CAR-T therapies and the model for distributing cold chain viral vector therapy including logistics, title transfer and reimbursement

7. Provided full-service investor relations support for wide range of cell and gene therapy companies

8. Developed medical science liaison content and materials for engagement with HCPs for CAR-T

Actively publish original rare disease and CGT research

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Routledge
Taylor & Francis Group

ORIGINAL RESEARCH ARTICLE

OPEN ACCESS Check for updates

Cost-effectiveness analysis of using onasemnogene abeparvocec (AVXS-101) in spinal muscular atrophy type 1 patients

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ABSTRACT

Background: Spinal muscular atrophy type 1 (SMA1) is a devastating genetic disease for which gene-replacement therapy may bring substantial survival and quality of life benefits.
Objective: This study investigated the cost-effectiveness of onasemnogene abeparvovec (AVXS-101) gene-replacement therapy for SMA1.
Study design: A Markov model was used to estimate the incremental cost-effectiveness ratio (ICER), expressed as cost/quality-adjusted life year ($/QALY), of AVXS-101 versus nusinersen over a lifetime. Survival, healthcare costs and QALYs were estimated using natural history data for SMA patients who achieved motor milestones (sitting/walking). Health utility weights were obtained from the CHERISH trial.
Setting: USA; commercial payer perspective
Participants: SMA1 infants
Interventions: AVXS-101 was compared to nusinersen.
Main outcome measure: The primary outcome was the ICER.
Results: Expected survival (undiscounted) over a lifetime predicted by the model was 37.20 life years for AVXS-101 and 9.68 for nusinersen (discounted QALYs, 15.65 and 5.29, respectively). Using a potential AVXS-101 price range ($2.5-5.0M/treatment), the average lifetime cost/patient was $4.2–6.6M for AVXS-101 and $6.3M for nusinersen. The ICER range was (-$203,072) to $31,379 per QALY gained for AVXS-101 versus nusinersen, indicating that AVXS-101 was cost-effective with prices of ≤$5M.
Conclusion: Single-dose AVXS-101 was cost-effective compared to chronic nusinersen for SMA1 patients.

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KEYWORDS
AVXS-101; cost-effectiveness; gene-replacement therapy; nusinersen; onasemnogene abeparvovec; spinal muscular atrophy type 1

Introduction

Spinal muscular atrophy (SMA) is an inherited neuromuscular disease with severity ranging from progressive infantile paralysis and premature death (SMA types 0 and 1 [SMA0 and SMA1, respectively]) to limited motor neuron loss and normal life expectancy (SMA type 4 [SMA4]) (Table 1) [1,2]. It is the second most common fatal autosomal recessive disorder after cystic fibrosis, with a prevalence of approximately 1–2 per 100,000 persons and incidence around 8 per 100,000 live births [2].

Infants with SMA1 often have onset of clinical signs during the first few months (usually within 4 to 5 months) of life, and fail to reach basic developmental motor milestones, such as the ability to sit without assistance. These infants experience rapid, significant and progressive muscle deterioration, leading to the inability to breathe or swallow, and these complications are the major cause of morbidity and mortality in SMA [1,3,4]. Survival is extremely poor; the median age of death or permanent ventilation is 10.5 months, and 92% will expire or rely on permanent ventilation by the age of 20 months [5].

Most forms of SMA are caused by the loss or mutation of the primary *survival motor neuron* (*SMN1*) gene, resulting in SMN protein deficiency [1]. Variability in the severity of disease is highly correlated with the copy number of the secondary *SMN* gene (*SMN2*). Although *SMN2* cannot completely compensate for the loss of the *SMN1* gene, patients with milder forms of SMA generally have higher *SMN2* copy numbers [6,7]. Patients with fewer than three copies of *SMN2* most commonly present as SMA1 (also named Werdnig-Hoffmann disease), which accounts for around 60% of all SMA cases [8,9].

In the USA (U.S.), SMA therapies were strictly supportive/palliative and did not preserve motor neurons or improve weak musculature until 2016 when nusinersen was approved by the U.S. Food and Drug Administration

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Meeting the affordability challenges posed by orphan drugs: a survey of payers, providers, and employers

Erin Lopata, PharmD, MPH; Christopher Terrone, MS; Ami Gopalan, PharmD, MBA, FAMCP; Nicholas Ladikos, PharmD, BCPS, BCGP, BCIDP; and Terry Richardson, PharmD, BCACP

What is already known about this subject

- Payers and employers have traditionally managed high-cost orphan drugs through utilization management strategies, with coverage and level of restriction varying across payers and employers.
- With a robust pipeline of orphan drugs anticipated to enter the market, including gene therapies and other high-cost, 1-time treatments, many health care stakeholders are questioning if they can afford to continue to cover them for their populations.

What this study adds

- The perspectives of a diverse group of health care stakeholders, including payers, employers, and providers, on current and future strategies to manage orphan drugs.
- As providers navigate payer coverage, benefit, and site-of-care requirements in the provision of care to patients with rare conditions, they are considering the cost burden on patients as well as the administrative burden related to the acquisition of orphan drugs.

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ABSTRACT

BACKGROUND: As an increasing number of orphan drugs are FDA approved, health care payers, employers, and providers are attempting to strike a balance between patient access to innovative treatments and overall affordability. Payers and employers are evaluating how traditional specialty pharmacy management strategies and innovative models can support continued coverage of orphan drugs.

OBJECTIVE: To understand how health care stakeholders are meeting the financial challenges posed by the increasing number and cost of orphan drugs and how these strategies are affecting orphan drug acquisition for providers.

METHODS: A survey was conducted with payer, provider, and employer decision makers recruited from both AMCP and a proprietary database of market-access decision makers in July and August 2020. Respondents were asked about their experiences and activities in the orphan disease space, including tactics to manage affordability of drugs to treat orphan diseases.

RESULTS: Reinsurance was the most commonly utilized strategy to maintain affordability of the benefit for both payers (42%) and employers (55%). Although 31% of payers have adopted gene therapy carve-outs, no employers had done so. Approximately three quarters (76%) of payers believe that limited distribution networks impede their abilities to manage orphan drugs, compared with 4% who believe limited networks improve orphan drug management. For most payers (78%), the decision to cover orphan drugs on either the medical or pharmacy benefit depends on the specific drug. Medical benefit coverage was driven primarily by site-of-care policies (55%) and the lower drug cost of average sales price pricing (50%). Pharmacy benefit coverage was driven primarily by a greater ability to manage the orphan drug (71%) and by rebates (62%). One in 3 (33%) of providers with experience treating orphan diseases acquire orphan drugs exclusively through buy and bill, whereas 10% acquire them exclusively through a specialty pharmacy provider. Buy-and-bill acquisition by providers was driven primarily by improved patient affordability (47%) and 340b pricing (47%). Specialty pharmacy

Costs and health resource use in patients with X-linked myotubular myopathy: insights from US commercial claims

Naomi C Sacks, PhD; Bridget E Healey, MPH; Philip L Cyr, MPH; Theodore Slocomb, MBA; Emma James, DPhil; Alan H Beggs, PhD; and Robert J Graham, MD

What is already known about this subject

- Most children with X-linked myotubular myopathy (XLMTM) depend on permanent use of mechanical ventilation, feeding tubes, and wheelchairs.
- The current costs of care for this rare disease have not been quantified.

What this study adds

- This is the first study that has attempted to quantify the economic burden of XLMTM in the United States and has found that annualized all-cause medical costs per patient totaled $897,978 in the first year of life and nearly $1.5 million total for patients who survived the first 4 years of life.
- This study supports the value of potential future treatment options for XLMTM and could inform the collection of economic impact evidence for other rare genetic diseases.

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ABSTRACT

BACKGROUND: In X-linked myotubular myopathy (XLMTM), mutations in the *MTM1* gene result in absence or dysfunction of myotubularin, a protein required for normal development, maintenance, and function of skeletal muscle. Extreme muscle weakness results in severe respiratory failure that is fatal for approximately half of XLMTM-affected children by age 18 months. Most surviving patients require invasive mechanical ventilation, feeding tubes, and wheelchairs for mobility, due to profoundly impaired motor function. Little is known about the costs of care for this rare disease. Currently, there are no approved therapies for XLMTM.

OBJECTIVE: To quantify the direct medical costs and health care resource utilization (HRU) incurred by XLMTM patients and paid by commercial insurers.

METHODS: A retrospective, longitudinal study was conducted using the IQVIA PharMetrics Plus commercial database of adjudicated claims for more than 140 million individuals with commercial insurance coverage in the United States. An algorithm based on demographic information, diagnosis and procedure codes, and medications was used to identify XLMTM patients younger than aged 2 years during the study period from January 1, 2006, through September 30, 2018. All-cause direct medical costs and HRU during each month were calculated. Costs were grouped as inpatient hospital

THANK YOU!

we look forward to discussing next steps

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