Pediatric-Friendly Orphan Medicines

September 12th 2018
Euronext: ADVIC
Investor Confcall
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Today’s speakers

Luc-André Granier, MD, PhD
Co-founder and CEO

Previously worked at:

Ludovic Robin, Pharm.D, MBA
Chief Business and Strategy Officer

Previously worked at:
Introducing Advicenne – Euronext: ADVIC

- Specialty pharmaceutical company focused on developing novel therapeutics for rare and orphan pediatric diseases with significant unmet medical needs
- KOL-driven research and development approach with a focus on nephrology and neurology
- Clinically de-risked: mature and balanced pipeline includes preclinical through late-stage assets (pre-registration, phase 3)
- Lead asset, ADV7103 for the treatment of distal Renal Tubular Acidosis (dRTA)
- Successful December 2017 initial public offering, raised $33.4 million*
- Well financed with cash and cash equivalents of $43.5 million at December 31st 2017*
  - Half-year results to be published next week

* € = $1.20
Recent Highlights

➢ ADV7103 for the treatment of distal Renal Tubular Acidosis (dRTA)
  ✓ On track for European market authorization filing end of the year
    • ADV7103’s safety and efficacy was confirmed after 24 months in the Phase III Extension Study – July 2nd 2018
  ✓ IND for US Phase III pivotal trial received – September 5th 2018

➢ ADV7103 for the treatment of Cystinuria
  ✓ Authorisation from French regulation authorities to initiate pivotal phase II/III trial of ADV7103 in Cystinuria – May 4th 2018

➢ ADV6209 (Ozalin®)
  ✓ Positive opinion from European National Authorities for ADV6209 in pediatric Moderate Sedation – September 12th 2018
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Source: Company information

ADV6209 – MAA approval
- Sold to Primex Pharmaceuticals in 2016
ADV6209 for Moderate Sedation

➢ ADV6209 under the brand name **OZALIN®** is indicated
  ✓ in children from 6 months to 17 years old,
  ✓ for moderate sedation before a therapeutic or diagnostic procedure or as premedication before anaesthesia.

➢ **Therapeutic objectives in paediatric patients**
  ✓ guard the patient’s safety and welfare;
  ✓ minimise physical discomfort or pain;
  ✓ control anxiety, minimise negative psychological trauma, and maximise the potential for amnesia;
  ✓ control behaviour and/or movement so as to allow the safe completion of the procedure; and
  ✓ return the patient to a state in which safe discharge from medical supervision.

➢ Total market potential\(^1\) estimated by Primex is $100 – 350m annual sales

Source: Company information
1: Primex information
An innovative oral solution of midazolam

- Significant unmet medical need in children
- Formulation initiated by Amiens’ University Hospital
- Improved palatability and exact dose adapted to child's body weight
- New route of administration

advicenne was in charge of

- Pharmaceutical and industrial process development
- Clinical development program and execution
- Regulatory strategy
- Regulatory submission to Health agencies

Source: Company information
ADV6209: Efficacy Results in Clinical Trial

Evaluation of the Efficacy of ADV6209 for Moderate Sedation in Pediatric Patients compared to Meta analysis of literature data

Types of EU marketing authorisation procedures

**Decentralized procedure**

- All other products

  - ADV6209 Ozalin®

  - One or more EU member states
    - Based on recognition by national authorities of a first assessment performed by one Member State

**Centralized procedure**

- only for orphan diseases, cancer and specific other indications

  - ADV7103

  - All EU member states

Source: Company information, EMA
ADV6209: Smart Development and Anticipated Success

➢ Decentralized procedure in November 2016
  ✓ Only EU acceptable procedure for such a product
  ✓ France, UK, Italy, Netherlands, Norway, Sweden, Finland

➢ Partnership with Primex Pharmaceuticals in February 2016
  ✓ Upfront payment
  ✓ Milestones and profit sharing, up to several tens of €m

➢ Pre launch ongoing

Source: Company information
ADV7103
1 Product for 2 Diseases, dRTA and Cystinuria

Two rare/orphan indications

Addressable population

- dRTA (genetic and acquired)
  - Approx. 30,000\(^1\)
  - Approx. 20,000\(^1\)

- Cystinuria
  - Approx. 70,000\(^2\)
  - Approx. 20,000 – 30,000\(^2,3\)

Significant unmet medical need

- Standard of Care requires 3 to 6 doses per 24 hours, resulting in sleep disruption
- Lack of compliance adversely affects treatment efficacy
- Direct impact on quality of life, especially for pediatric patients

1: Company estimates based on the low range prevalence considered by the EMA for the ODD.
2: Eggermann T. and al, Cystinuria: an inborn cause of urolithiasis, Orphanet Journal of Rare Diseases 2012; 7:19
3: NORD cystinuria
dRTA and Cystinuria: 1 Product for 2 Diseases

Market: High demand with few competitors

- **No approved** treatment
  - dRTA: SoC requires compounding various unapproved products in an attempt to re-establish normal physiological functions
  - Cystinuria: SoC is diet, hyperdiuresis and compounding of various alkalising unapproved products administered every 4 to 6 hours
- SoC induces **severe complications** in the gastro-intestinal tract
- Not adapted for **pediatric use**

ADV7103 close to market for dRTA (1 year-lag for Cystinuria)

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<tr>
<th>Event</th>
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<td>MAA submission</td>
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<td>Core market access dossier</td>
<td>2018 – 2019</td>
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<tr>
<td>FDA filling and review</td>
<td>2019 – 2020</td>
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<tr>
<td>Product launches</td>
<td>Without negotiation 2020</td>
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<tr>
<td>Finalise FDA discussion &amp; ODD</td>
<td>2018</td>
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<tr>
<td>Pivotal trial</td>
<td>2018 – 2019</td>
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<tr>
<td>Build commercial organization</td>
<td>EU 5 2019-2020</td>
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<tr>
<td>Launch product</td>
<td>2021/2022</td>
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1: Marketing Authorization Approval (MAA)
Source: Company information
Commercial Strategy

Market access strategy

- **Build a robust pharmacoeconomic core dossier to support orphan drug pricing of ADV7103 (EU and US) for both indications**
  - Target population
  - Value proposition
  - Pricing strategy

- **Build commercial organization in EU5 (France, Germany, Italy, Spain, United Kingdom)**
  - Limited prescribing centers
    - Develop KOL relationships
    - Communicate among the specialist community
  - Limited marketing and sales resources required: 4/6 sales reps per country
  - Good overlap between dRTA and Cystinuria prescribers
  - Establishes platform for additional products

- **Establish partnerships to generate sales outside the EU5**
  - US: Clinical development and registration by Advicenne, commercialized by partners
  - Other EU countries: European Market Authorization by Advicenne, commercialized by partners
  - RoW: Market authorization and registration by partner

Source: Company information
dRTA – Update for the next coming months

- On track for preparation of EU dossier for submission
- B21CS and B22CS clinical studies are completed
- Initiation of a 400-patients registry with ESPN* to understand disease natural History and epidemiology

- Structuration of US clinical operations
- IND opened on Sept 5th 2018.
  - Protocol available on clinicaltrials.gov
- First patient inclusion expected before the end of the year

Source: Company information
*ESPN: European Society for Pediatric Nephrology
Upcoming Value-Creating Milestones

**ADV7103 dRTA**

- **Orphan drug designation (ODD) in the US**
- **Start US pivotal Phase III trial**
- **Marketing Authorization Application (MAA) filling EU**

**ADV7103 Cystinuria**

- **Start European pivotal Phase II/III study**

**2018**

- **European Marketing Authorization Application (MAA) granted (Between End 2019/H1 2020)**

**2019**

- **Conduct and potential completion of European pivotal Phase II/III trial**
- **Orphan drug designation (ODD) granted in US and EU**

**2020**

- **Completion and data from the US pivotal Phase III trial**
- **New Drug Application (NDA) filing US**
- **Commercial launches EU**
- **Data from European pivotal Phase II/III trial**
- **Marketing Authorization Application (MAA) filling EU**
- **Start US pivotal Phase II/III trial**

Source: Company information
Corporate Highlights

1. De-risked Product Portfolio
   - Positive Phase III for lead product ADV7103
   - Strong clinical data demonstrates superiority of ADV7103 in dRTA as compared to Standard of Care
   - IND

2. “Niche-buster” Potential
   - ADV7103 is a pediatric-friendly treatment for two orphan nephrology indications
   - “Niche-buster” potential in well defined populations with no approved treatments in dRTA
   - ADV7103 to drive rich value-generating news flow over the next 18-24 months

3. Proven and Unique Strategy
   - Unique R&D approach driven by input from Key Opinion Leaders
   - Track record of successful product developments
   - Focus on pediatric-friendly treatments suitable for patients of all ages

4. Experienced Management Team
   - Experienced management team bringing years of drug development/regulatory experience

Source: Company information
Thank you

www.advicenne.com