# Pathbreaking treatments for rare diseases

Euronext: **ADVIC |** Nimes, France

Dag Van De Tips– ICC Ghent
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# Our mission

*“Advicenne is committed to developing and commercializing medications adapted to both children and adults*

*because pathbreaking treatments for rare diseases should be available to patients of all ages”*

# Experienced management team

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

Previously worked at:

Lilly

**Caroline Roussel-Maupetit, Eng**
Co-founder and
Chief Operating Officer

Previously worked at:

synthem

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

Previously worked at:

Shire

**Paul Michalet, MBA, CEFA**
Chief Financial Officer

Previously worked at:

metabolic explorer

Fermentalg

# Our cash-efficient business model

## From inception (2007) to 1st of January 2019

**~€30m invested**

### MEDICAL NEEDS FOCUSED
*Tailored approach*

- **KOL driven with direct feedback from physicians**
- **Patient centric development**
- **Innovative drugs from known APIs**

### TANGIBLE DEPLOYMENT
*Orphan & pediatric indications*

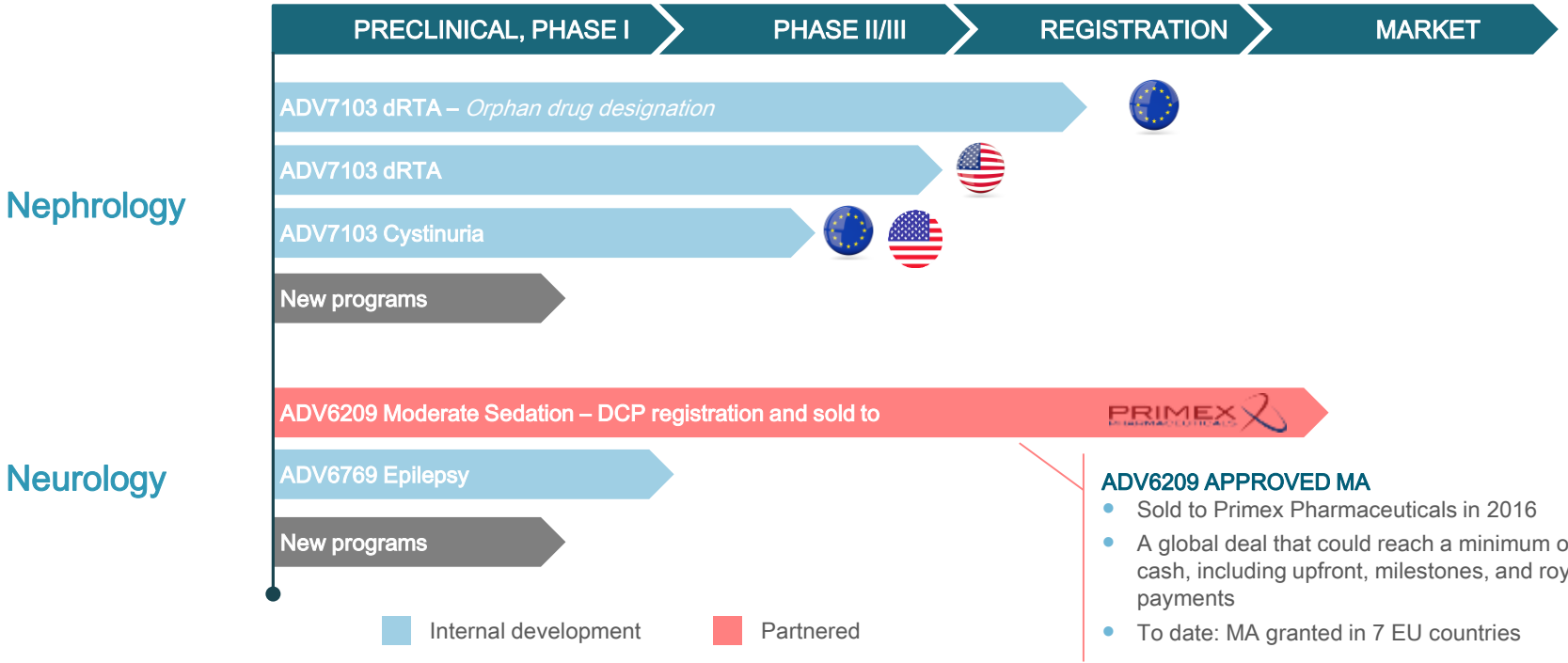
- **1 product already registered:** ADV6209 in Pediatric Moderate Sedation (EU)
- **2nd MAA pending:** ADV7103 in dRTA in (EU)
- **3 phase III studies ongoing:** ADV7103 in dRTA (EU & US) & in Cystinuria (EU)

### BUSINESS ORIENTED
*Partnerships*

- **A deal of up to €40m with Primex Pharmaceuticals on ADV6209 in 2016** (upfronts + milestones + royalties)
- Commercialized products (France)
- Worldwide flagship product commercialization strategy with EU5 direct sales organization

## A unique track record of efficient drug development

# Mature and balanced pipeline




**ADV6209 APPROVED MA**

- Sold to Primex Pharmaceuticals in 2016
- A global deal that could reach a minimum of €40m* in cash, including upfront, milestones, and royalty payments
- To date: MA granted in 7 EU countries

** $44.8m with 1€ = $1.12*

# ADV7103
## Global Development for dRTA

EU & US

# ADV7103 demonstrates superiority over SoC

- **Study results demonstrate significant efficacy**
  - Non-inferiority is clearly demonstrated on primary endpoint
  - ADV7103 is significantly superior to SoC on primary endpoint
    *P-value = 0.0047 (Per Protocol) – 0.0032 (Intention to Treat)*
  - Increasing responder rate with ADV7103
- **Efficacy was maintained after 24 months of treatment**
  - Blood bicarbonatemia in normal range in 79% of the patients
- **Excellent safety profile**
  - Only 11% of adverse events were potentially related to treatment, all of mild intensity
- **Strong improvement of quality of life over 24 months**
- **Strong compliance observed**
  - 93.3% and 89.6% of patients treated with ADV7103 were compliant after 3 and 6 months, respectively


# dRTA development plan in the US

- **Initiation of US clinical operations in 2018**
  - Dr Linda Law, appointed US VP Clinical Development and Medical Affairs
  - Two project leaders appointed
  - CRO to monitor the study in the US
- **IND clearance in September 2018**
- **Health Canada clearance in October 2018**
- **Study open and currently recruiting**

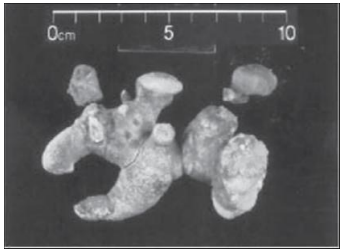
- **One pivotal Phase III study in the US required by the US FDA in addition to EU clinical package for registration**
- **B23CS : Pivotal study in US & Canada**
  - 3 to 5 central sites, 12 to 18 recruiting centers to be opened
  - 40 patients to be included

# ADV7103 for Cystinuria

EU

# ADV7103 : a second indication - Phase III - cystinuria

## Frequent Kidney Stones




*An inherited autosomal recessive disease characterized by the inability of the kidney to reabsorb cystine*

**Major** Complications

**Hypertension**
**Urinary tract infections**
**Renal impairment**
**Renal failure**

## Positive clinical proof of concept for Cystinuria

- Stabilizes urinary pH with only 2 doses per day
- Significantly increases pH level with a positive dose-response
- Strong supportive information linking increase of pH with solubility of cystine

## A pivotal Phase III study agreed to with EMA to support registration in EU

- Study ongoing in FR, BE
- To be opened in the UK
- 72 patients to be included

## US Strategy under review

- ODD to be submitted soon
- Meeting with FDA before year end

Source: Company information, Orphanet: cystinuria, NORD cystinuria, Eggermann T. and al, Cystinuria: an inborn cause of urolithiasis, Orphanet Journal of Rare Diseases 2012; 7:19

# ADV7103 Market potential

EU & US

# One product for 2 diseases: dRTA & cystinuria

## Two rare/orphan indications

| Addressable Global 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 needs

- Unregistered Standard of Care (SoC) 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: Low range prevalence considered by the EMA for ODD (EU/3/17/1888)
2: Eggermann T. and al, Cystinuria: an inborn cause of urolithiasis, Orphanet Journal of Rare Diseases 2012; 7:19

3: NORD cystinuria
Source: Company information, ODD (EU/3/17/1888), European Medicines Agency, U.S. National Library of Medicines

# One product for two diseases : dRTA & cystinuria

- **No approved first line treatment**
  - dRTA: SoC requires compounding of 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**
- **Poor compliance**

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


1: Marketing Authorization Application (MAA)

# Commercial strategy

## European approval and first product launches

- 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 i**n EU5 (France, Germany, Italy, Spain, UK)
  - 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
  - Establish platform for additional products

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

# Financial highlights

- Approximately € 22 million* ($24 million) in cash and cash equivalents as of June 30, 2019
  - €27 million ($31 million) raised in successful IPO in December 2017
- Streamlined operations with a headcount of 32 (21 in R&D)
- Cash sufficient to fund operations through numerous value-creating inflection points in the next 18 months
- € 20 million debt facility authorization from EIB (July 2019)

# Euronext: ADVIC

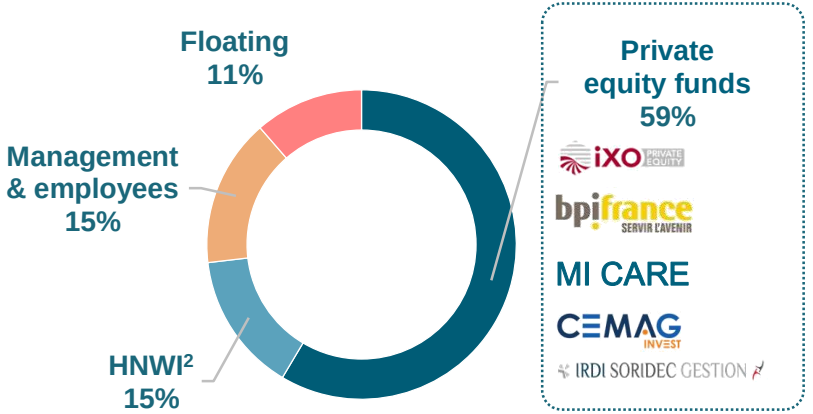
## COMPANY OVERVIEW

- Specialty pharmaceutical company
- Headquarters in Nîmes, France
- Founded in 2007
- Number of shares: 8,087,654
- Financing:
  - Approx. €30m in private rounds
  - €27.8m at listing on Euronext Paris in 2017
  - €20m loan facility from EIB, not yet drawn
- Cross listing on Euronext Brussels on June 12, 2019

## ANALYSTS COVERAGE

- France - Jamila Elbougrini (FR)
- The Netherlands - Anita Ye & Dylan van Haaften (ENG)
- UK - Samir Devani (ENG)

## SHAREHOLDERS AND INVESTORS[1]




*1: On a fully diluted basis as of June 30, 2019*
*2: High-net-worth individuals*
*Source: Company information*

# Thank you

September 2019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