



**ORPHAN NOW**  
turning no into now

Prepared by:  
Orphan Now Team

# Capabilities Presentation

**Providing Experience and Networks to Boost Rare Disease Companies' Performance**

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# What We Do

Accelerating meaningful benefits for patients living with rare diseases (RD) by supporting orphan drug (OD) companies



- ➔ Orphan Now is a patient-centric organization that guides and supports OD companies in their quest to develop and commercialize therapies to address the needs of patients suffering from rare diseases
- ➔ Orphan Now is a team of RD highly experienced senior leaders and functional experts ready to serve clients to:
  - **support clinical development and regulatory affairs efforts**
  - **define market entry strategies for fast orphan drug product launches**
  - **provide in-market capabilities for swift operational support and early revenue generation**
  - **give access to a broad and deep network of RD medical practitioners, leading hospitals, payors, PAGs and governmental authorities**
  - **generate and execute business development RD asset transactions**

# Who We Are

## Best-in-Class Orphan Drug Business Track Record



- ➔ Our team of senior rare disease business leaders and experts combines more than 200 years of orphan drugs' business success globally
- ➔ We offer very strong experience, know-how and networks in the rare disease space globally including all key markets
- ➔ We bring a proven track record on a range of functional areas:
  - Global and Regional leadership
  - General Management
  - Business Development transactions
  - Clinical Operations, Regulatory, Governmental Affairs, Patient Advocacy, Medical Affairs, Market Access, Marketing and Commercial Operations
- ➔ We have consistently delivered transformative results to more than 80 rare disease client companies since our establishment in 2014

# Who We Are - Team

Former leaders at Alexion, Celgene, Genzyme, Orphan Europe and Shire among other RD companies



**Franck Auvray**  
Partner

PharmD. - Rouen University  
MBA - Rouen Business School  
Over 25 years of pharma/biotech experience



**Jordi Badia**  
Founding Partner

JD – Barcelona University  
MBA – Indiana University / Kelley  
School of Business at Bloomington  
Over 25 years of pharma/biotech experience



**Mark Barrett**  
Partner

University of Hertfordshire  
Joint honours degree – Human Biology  
and Psychology  
Over 30 years of pharma/biotech experience



**Alain Coppex**  
Partner

Pham D. – Lausanne University  
Over 20 years of pharma/biotech experience



**Catharina Järnhem**  
Partner

BA Arts, Lund University  
Over 20 years of pharma/biotech experience



**Hans Peter Halbritter**  
Partner

MSc in Biology - Ludwig-  
Maximilians-University / Munich  
Over 30 years of pharma/biotech experience

# Who We Are - Team

World-class scientific, medical and legal expertise



**Anders Kärnell**  
**Partner**

MD, PhD – Karolinska Institutet, Stockholm  
MBA – Stockholm School of Economics  
Over 20 years of pharma/biotech experience



**Robert-Jan Van Son**  
**Partner**

MD – Utrecht University  
Over 25 years of pharma/biotech experience



**Marco Bocci**  
**Partner**

PhD - Cambridge University, UK  
DPharm - University of Siena, Italy  
MSc - Chemistry University of Siena,  
Over 20 years of pharma/biotech experience



**Ramon Vilalta**  
**Partner**

MD, PhD – Barcelona University  
Over 28 years of clinical experience in pediatric nephrology  
Extensive experience managing rare disease pediatric patients



**Alexander Natz**  
**Associated Partner**

JD – Bonn & Duke Universities  
Attorney at Law  
Over 20 years of pharma/biotech experience

# Who We Are: World-Class Strategic Partnerships to Drive Value

Global reach through strategic partnerships



## Rapid Commercialization Partners

Optimizing commercial readiness to drive value, accelerate time to market and maximize revenue potential.

Based in the Boston - USA



## Seventeen Pharma

Seventeen Pharma is supporting biotech companies to develop and execute their commercial plans for Switzerland.

seventeen  
p h a r m a

Based in Lausanne - Switzerland

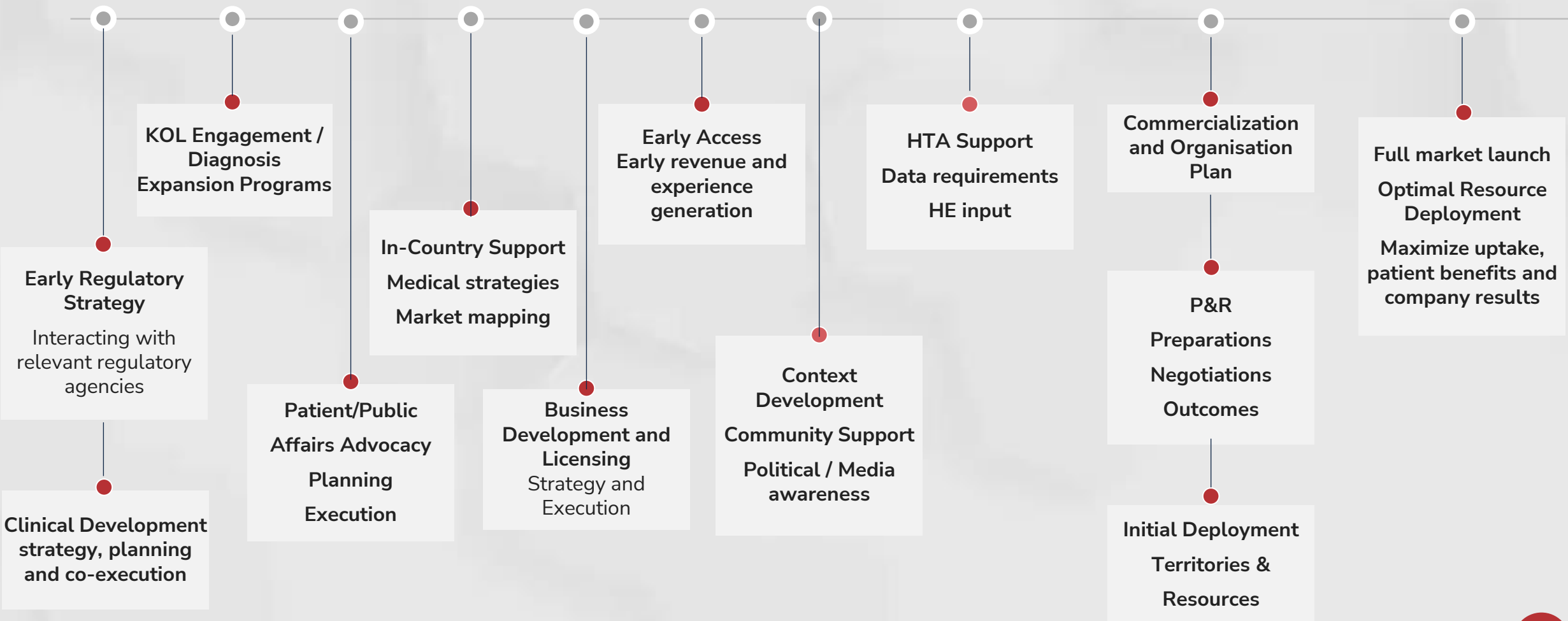
Additionally, Orphan Now counts on a best-in-class list of external advisors from the rare disease patient advocacy space, leading university hospitals, the venture capital community and the investment banking industry

# Ensuring RD Clients' Success at Every Stage

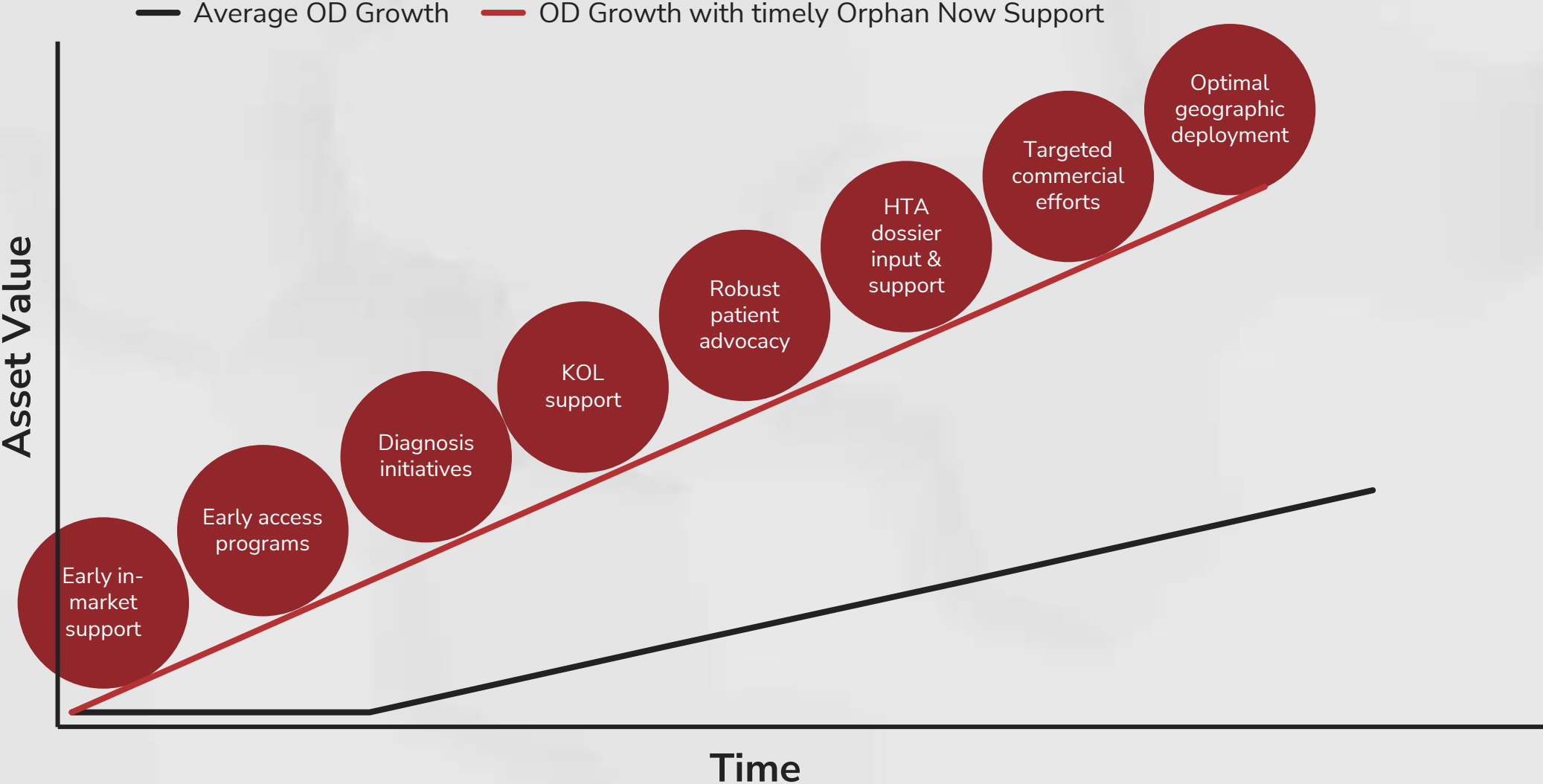
## Development & Filing

## Access

## Go-to-Market



# Ensuring RD Clients' Success at Every Stage: OD Launches



# OD Product Launches: Responding to Critical Challenges

1

## Maximizing the market opportunity

What is the best regulatory strategy for any given jurisdiction?

Which clinical trials will enable wide labels and fast approvals?

What is the size, value and shape of the opportunity in the key markets?

Where are the largest groups of existing diagnosed patients?

Which are the best patient identification and diagnosis expansion programs to maximize the opportunity?

2

## Identifying and mapping key centers and KOLs

Which countries, centers and investigators should be contacted for clinical trials?

Who are the active KOLs in the therapeutic space and how can they be brought onboard?

What is the best strategy for reaching out to the key players in the medical community?

3

## Designing effective market / patient access strategies

Who are the government agencies and decision makers relevant to each client/country?

What are the best EAP, PA, GA and P&R combined strategies to maximize patients' benefits and early revenue?

With whom, how and by when should stakeholders' engagements be initiated?

Which are the most appropriate pricing corridor strategies in view of the relevant landscapes?

4

## Defining and executing go-to-market strategies and tactics

What are the options, risks and benefits of "going it alone" vs. partnering vs. asset selling in key territories?

Which in-market resources are needed and what is the best sequence for fast, and enduring global revenue and profit?

Which commercialization plans and tactical programs will maximize the value of the opportunity?

# The “ONE” platform program

## Market Challenge

US rare disease companies approaching new orphan drug business opportunities must make critical decisions on how to succeed in their second most important market – Europe<sup>1</sup>

## Solution – the “Orphan Now Europe” platform program (ONE)

The ONE platform program is the best available solution to US rare disease companies for entering the European rare disease market. The success of the ONE platform program is based on Orphan Now’s team members European market entry proven track record established while holding European leadership roles at Alexion, Celgene, Genzyme, Orphan Europe and Shire, among others. The ONE platform program has enabled successful European market entries to many Orphan Now client companies over the last decade

1. Orphan Drugs Market Size, Share & Forecast, 2022 to 2032. Future Market Insights Report, 2022.

# The “ONE” platform program

## Why is the ONE platform program so successful for Orphan Now clients?

Because this program is a temporary “plug-and-play” solution to swiftly capitalize on OD business opportunities in Europe:

- ✓ It allows to make well-informed decisions on European market entry strategies: “go-it-alone” vs licensing vs hybrid models
- ✓ It enables to jumpstart orphan drugs’ European operations utilizing a well-established orphan drug business platform
- ✓ It de-risks European market entries by creating a full operational platform without costly long-term commitments
- ✓ It provides safe grounds for sound and timely European investment decisions
- ✓ It buys precious time to early unlock the value of orphan drugs assets in Europe
- ✓ It ensures a smooth, timely and flexible operational transition from the ONE platform to the client’s European operational infrastructure according to the needs of the client
- ✓ It is established as Orphan Now’s most successful offering thanks to our client's word-of-mouth: the ON team has consistently and successfully delivered ONE platform program solutions to clients many times over the last 10 years

# The “ONE” platform program

## What is the ONE platform program?

It is a fully flexible and ready-to-use solution that enables rare disease companies to enter European markets early and quickly. It provides guidance and support in critical business areas - client companies pick and choose from a 360° array of options:

- ✓ Engaging with regulatory authorities to accelerate MA approvals
- ✓ Mapping the landscape across markets to determine the size and shape of each opportunity
- ✓ Identifying and interacting with relevant sites for patient enrolment in clinical development and in early access programs
- ✓ Providing SCM and pharmacovigilance solutions for clinical development and/or for commercial efforts
- ✓ Developing launch strategies including country entry sequencing for optimal deployment of resources and revenue generation
- ✓ Assessing and implementing pricing and reimbursement strategies to maximize the value of the opportunity
- ✓ Generating and managing early access programs - enabling prompt patient access to needed therapies, early therapy experience for clinicians, and swift revenue generation for companies
- ✓ Having “boots on the ground” to execute pre-launch and early commercial activities to maximize penetration speed and revenue
- ✓ Supporting companies in their efforts to build their own European infrastructure and / or to establish partnerships

# Some of Our Client Companies

Aegerion<sup>®</sup>  
Pharmaceuticals

ultragenyx  
pharmaceutical

B:OMARIN<sup>®</sup>

Jazz Pharmaceuticals<sup>®</sup>

Orchard  
therapeutics

avexis

clementia

gsk  
GlaxoSmithKline

NPS Pharma<sup>™</sup>

SUCAMPO

biocryst

OxThera

Almirall

Chiesi

ISCONOVA

Ablynx

H HORIZON  
P H A R M A

AKCEA<sup>®</sup>  
THERAPEUTICS

Pfizer

raptor  
pharmaceutical corp.

Medivir

HANSA<sup>®</sup>  
BIOPHARMA

# Summary

**At ON, we turn  
NO into NOW  
for rare disease  
patients and  
orphan drug  
companies<sup>®</sup>**

Laser-focused on rare disease patients and orphan drug companies

Highly successful team with unparalleled RD experience and networks

“ONE” program: “plug-and-play” solution to enter / operate in Europe

Consistent delivery of impactful results for client companies since 2014

Sense of urgency: in rare diseases, every patient counts... every day

# Contact Details



**ORPHAN NOW**  
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At Orphan Now, we turn NO into NOW  
for rare disease patients and orphan  
drug companies®



## Email

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[info@orphannow.com](mailto:info@orphannow.com)



## Website

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[www.orphannow.com](http://www.orphannow.com)



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# Thank You

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