



# Open Orphan

**Open Orphan plc**

March 2022

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**World leader** *in*  
***testing infectious & respiratory  
disease products*** *using human  
challenge studies addressing the  
growing infectious disease  
market*

# Highlights

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- ✓ Subject to audit:
  - Expect full year EBITDA profitability FY21
  - Expect c. £40m revenue for FY21
  - Cash & cash equivalents 31 December 2021: £15.6m (30 June 2021: £14.9m)
  - Targeting c. £50m non-Covid revenue FY22
    - Any COVID-19 revenue will be in addition to this
- ✓ Bed capacity increase to 62 from 43 on a low cost basis
- ✓ Strong pipeline of challenge studies
- ✓ Continuing to work towards monetisation of non-core assets



# A Fast Growing Business



£46.6m

Disclosed contract wins  
2021

7

Challenge study  
contract wins 2021

£75m

Current Pipeline (Weighted)  
BD Pipeline X Probability of Award



**Expansion** To facilitate growing pipeline & new service offering

**FluCamp**  
Clinical Trials Recruitment

- 📍 QMB: 24 + 7 beds
- 📍 Whitechapel Clinic: 19 beds
- 📍 Plumbers Row: 12 beds
- 📍 Manchester

62 beds	New Manchester Facility 4,000sq ft	Plumbers Row office + screening site 9,000sq ft
Capacity increase by c. 45%	Old Manchester Facility 500sq ft	Old Alie St. office (opened by previous management) 4,000sq ft
	Same cost	1/3 <sup>rd</sup> cost

# Monetisation of Non-Core Assets



**COMPLETED**

- In June 2021, the first distribution in specie was handed back to ORPH shareholders
- Created substantial value for Open Orphan shareholders

**PrEP**Biopharm

62.6%  
stake

- Viral prophylactic
- Phase II nasal spray solution
- Sale to a Nasdaq cash SPAC aborted
- Optimistic new sale can be completed

**IMUTEX**

49%  
stake

- FLU-v, Phase III ready universal flu vaccine
- AGS-v, Phase II ready universal mosquito saliva vaccine
- Still pushing management team of ConserV Bio to help sell 100% Imutex

**Disease in Motion®**

100%

- Worlds largest database of infectious disease progression data
- Potential applications across a wide variety of end users (big tech, wearables, pharma and biotech)

## Benefits of Spin Off's

- ✓ Cost synergies / savings
- ✓ Creating value where previously none
- ✓ Dividend for ORPH shareholders
- ✓ Removing from balance sheet
- ✓ Opportunity for assets to be developed

# New CEO

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## **Yamin 'Mo' Khan appointed as CEO on 24 February**

- Cathal Friel will remain hands-on as Executive Chairman

### **Biotech Experience**

- The Liposome Company

### **Extensive CRO experience (25+ years)**

- Innovex – acquired by Quintiles
- Pharm-Olam – acquired by PE
  - FTE expansion from 20 FTEs to 600+ FTEs
  - 2 countries to 40+ countries

### **Commercial Management – experience**

### **Operational Management – experience**



# Open Orphan – at a Glance

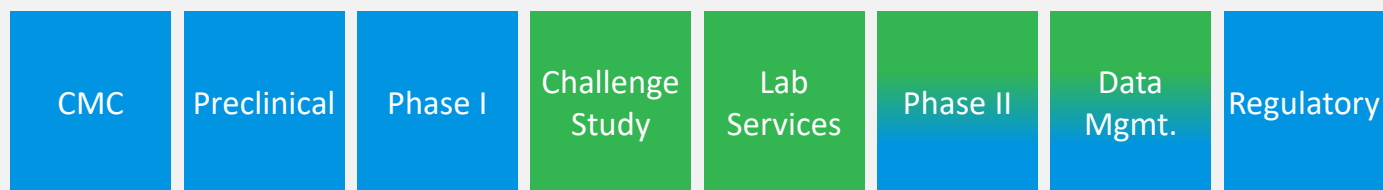


## 1 Undisputed Leader in Challenge Studies

- ✓ 30+ years providing world leading clinical trials service
- ✓ 60+ challenge studies completed
- ✓ 3,300+ patients inoculated
- ✓ Significant long-term projects: £5-10M+ typical study size across 8-10 months average study duration
- ✓ World's largest portfolio of human challenge models
- ✓ Large pharma & smaller biotech client base
- ✓ Deep roots dating back to 1947 with the establishment of the UK Common Cold Clinic (and continuous support from the UK government since)
- ✓ The Company's challenge studies are subject to the most rigorous external regulatory and ethical review
- ✓ WHO guidelines on Challenge studies
- ✓ In 60+ challenge studies completed by the Company, all have recruited successfully

## 2 A Comprehensive Suite of Services...

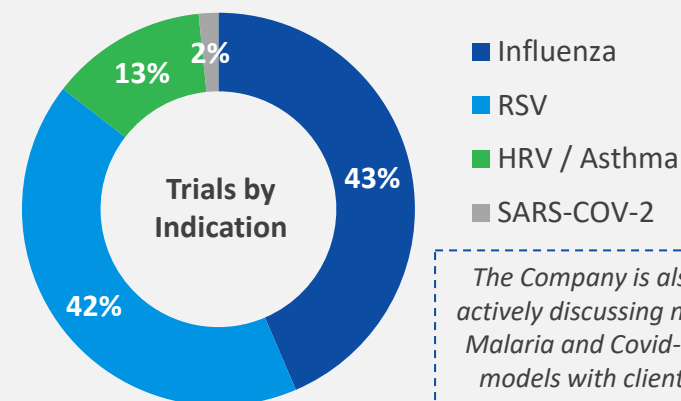
● hVIVO Capability ● Venn Life Sciences Capability



## 3 Human Challenge Trials Completed

Challenge Model	# of Trials	# Patients Inoculated
HRV / Asthma	8	373
Influenza	27	1,399
RSV	26	1,532
SARS-COV-2	1	36
<b>Total</b>	<b>62</b>	<b>3,340</b>

## 4 Experienced Across Various Indications



# Open Orphan- at a Glance



- Open Orphan Location
- hVIVO Location
- Venn Location

Overview of the Company's Facilities		
	Name	Description
1	Dublin Office	Flexible shared offices
2	Manchester	Recruitment & vaccination site
3	BioBank	Storage unit for biobank
4	Plumbers Row*	Office space; volunteer screening; 12-bed unit
5	Whitechapel Clinic	19-bed unit; 10 screening, admin & storage rooms
6	Queen Mary's BioEnterprise Centre (QMB)	24 + 7 bed unit; virology laboratory
7	Venn (Breda)	Flexible shared offices
8	Venn (Paris)	Flexible shared offices

\*As of 21 March 2022



# Differentiated Volunteer / Patient recruitment



## FluCamp

### Clinical Trials Recruitment

- ✓ Decades of experience attracting suitable healthy subjects to meet recruitment requirements (primarily sourcing subjects for hVIVO trials to date)
- ✓ Leading tech-enabled screening currently pivoting the platform to find patients (as well as healthy volunteers)

- ✓ *Central London and Manchester screening clinics provide a market leading advantage in recruiting healthy subjects and patients for a broad range of trials amongst diverse population bases*
- ✓ *Provides ability to recruit efficiently for the significant increase in respiratory & infection disease field trials in a timely, cost-effective manner*
- ✓ *100% success*

#### **Opportunity #1**

*Ability to sell our 160k annual leads to other Phase 1 units*

#### **Opportunity #2**

*Opportunity to support the recruiting for Phase 2/3 trials for a strategic partner*

30 +

Year History

250,000 +

Active Subjects in Existing Database

160,000 +

New Leads per year through FluCamp.com

1,000+

Weekly Screening  
Capacity

~85%

of subjects screened for human challenge  
trials can be utilised for other trials

# Why Challenge Studies?



- Potential for **fast track / break through designation**
- Rapid **approval or rejection** of pre-clinical data
- Phase II entry - up to **5x valuation** uplift
- Significant **time / financial savings** on efficacy data vs field-based Phase II
- **Facilitates dose ranging studies** to support efficacy
- Expedites important **“Go/No-Go” decisions**
- Generate **invaluable safety & efficacy data** to optimise Phase III program
- **Reduce no. patients** required for Phase III
- Identify **key biomarkers**
- Test for efficacy against specific **variants**
- Investigate **key scientific questions** for particularly novel products, mechanisms and approaches
- Potential **approval and emergency use** authorization

*“Pfizer jumps the queue and leaps into RSV vaccine contention with human challenge trial”*



**FDA Breakthrough Therapy Designation Granted for Respiratory Syncytial Virus Vaccine**



*the*pharmaletter

Bavarian Nordic gets Breakthrough status for its RSV vaccine candidate

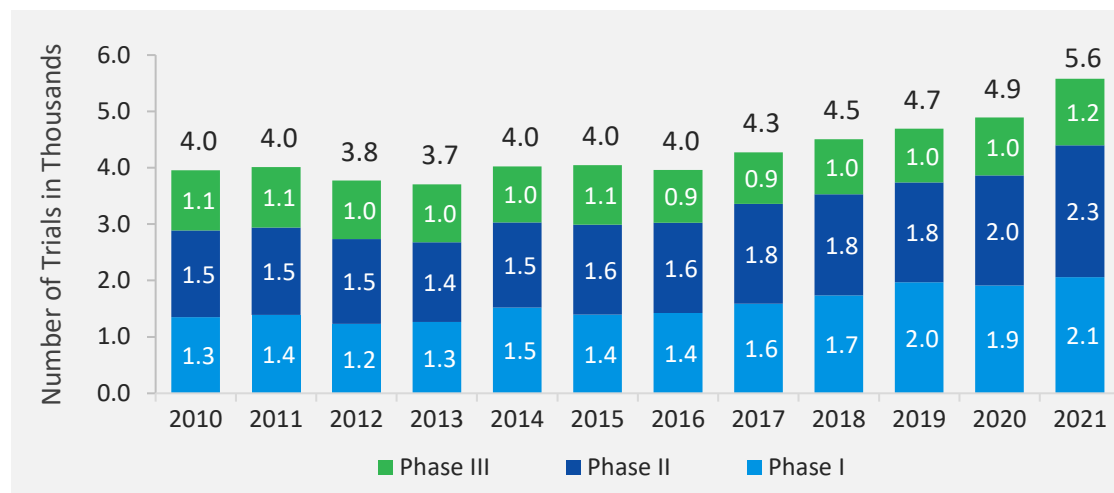


*human challenge trials & immunogenicity studies can play an important role in accelerating and improving vaccine testing and development in a pandemic*

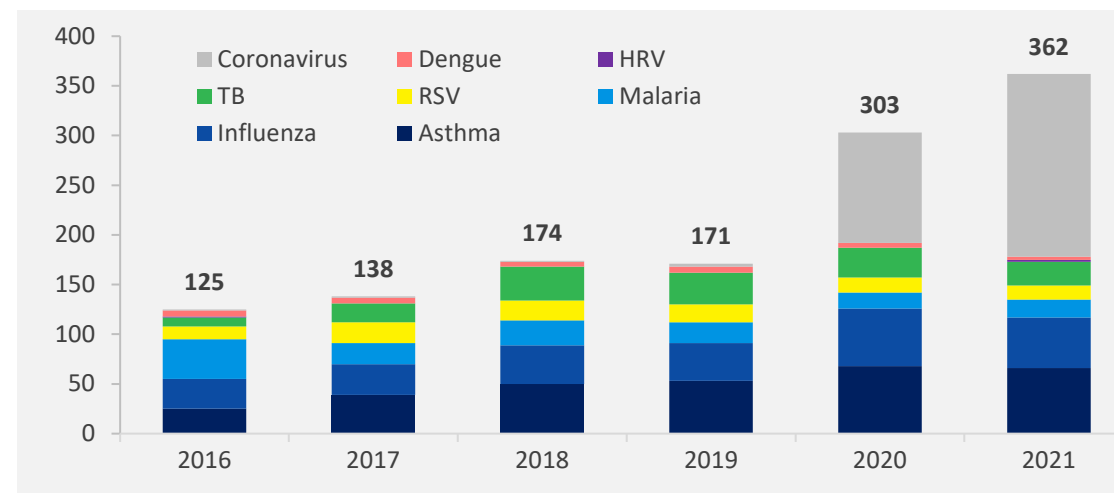
# Potential Market



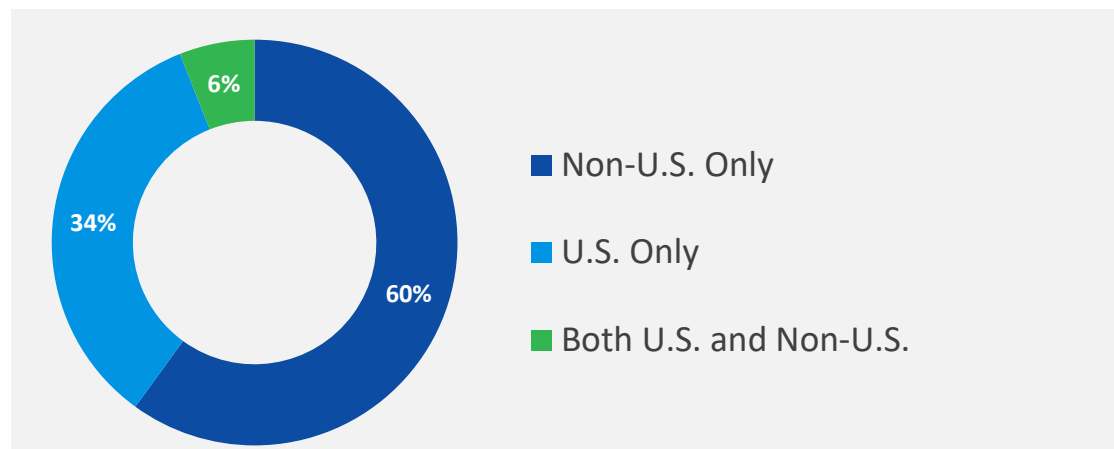
**Strong Growth in Phase I and II Clinical Trial Starts<sup>(1,2)</sup>**



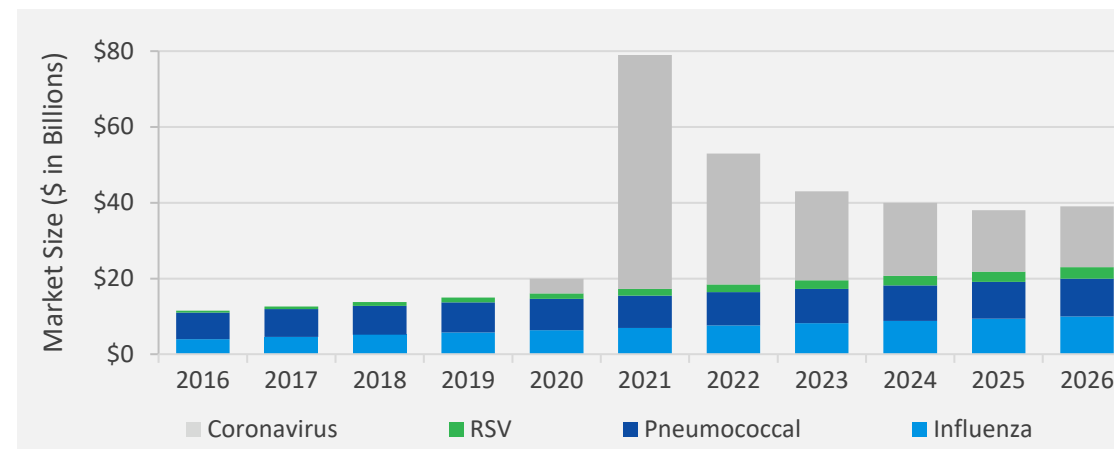
**Increase in Phase I & I/II Infectious Disease & Respiratory Trials<sup>(3)</sup>**



**Global Clinical Trials Market<sup>(1)</sup>**



**Strong Growth Forecasted for Anti-infective & Vaccine Indications<sup>(1)</sup>**



Note (1): Source: Citeline Trialtrove, Jan. 2022 and Pharma R&D Annual Review; IQVIA Institute, Global Trends in R&D – Overview Through 2021; ClinicalTrials.gov; Evaluate Pharma; Edison Investment Research.

Note (2): Phase II includes Phases I/II, II, IIa, IIb. Phase III includes Phase II/III and III. Trials were industry sponsored, interventional trials (device trials were excluded).

Note (3): Source: Citeline Trialtrove, Jan. 2022 and Pharma R&D Annual Review; IQVIA Institute, Global Trends in R&D – Overview Through 2021; Global Data; Evaluate Pharma; Edison Investment Research; Pitchbook

# Expanding our Capacity & Service Offering



## Capacity & offering

- ✓ New facilities in London & Manchester
- ✓ Supplemental recruitment services
- ✓ Non- first in human Phase I trials
- ✓ Field-based Phase II trials
- ✓ Clinical Site
- ✓ Provision of healthy volunteers
- ✓ Increase in capacity
- ✓ Cross-selling opportunities



## Challenge study models

- ✓ STRiVE Project
- ✓ Malaria
- ✓ COVID-19
- ✓ Exploring more







## Laboratory facilities & services

- ✓ New facilities
- ✓ Increased biomarker & molecular testing capabilities
- ✓ Virus manufacturing
- ✓ Lab services are progressing towards CAP and UKAS accreditations

# Open Orphan – By the Numbers



 <b>Rapidly Growing Specialist CRO</b>	<b>c. £40M</b> 2021P Revenue	<b>c. £50M</b> 2022P Non-Covid Revenue	<b>£15.6M</b> Cash at year end 31 December 2021
 <b>Leader in Human Challenge Trials</b>	<b>60 +</b> Completed Human Challenge Studies	<b>9+</b> Challenge Study Models	<b>£50M +</b> Challenge Model Portfolio
 <b>Operational Excellence</b>	<b>84k +</b> Volunteers screened 2021	<b>£5-10M</b> Typical Study Size	<b>8-10 mos.</b> Average Study Duration
 <b>Well Positioned for Growth</b>	<b>62 +</b> c.45% increased bed capacity	<b>86k +</b> Lab samples analysed 2021	<b>£75M +</b> Current Pipeline (Weighted)*

\*Weighted Pipeline = BD Pipeline X Probability of Award



# Open Orphan

## Questions



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