

Fundify

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January 2022



Katya Tsaoun, Ph.D.

Founder and CEO

[Katya@Akttyva.com](mailto:Katya@Akttyva.com)

617-821-7095

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# Company Highlights

## Company Overview

- Founded in 2020
- Experienced team with extensive biology, drug discovery, development, and commercialization expertise
- Focus: endothelial vascular leak
- **National Science Foundation Award recipient December 2021**



## Pipeline

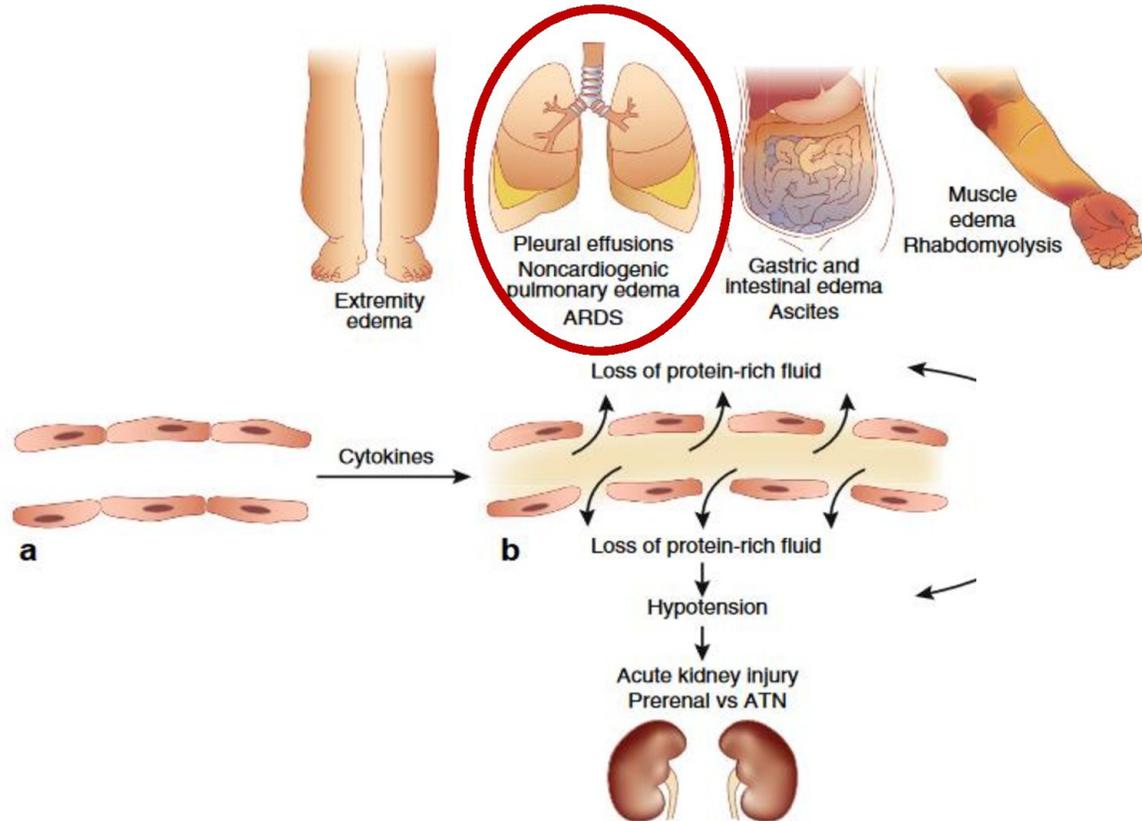
- Lead program: AKT-001 as a treatment for ARDS
- Additional potential indications: diabetic macular edema, systemic capillary leak syndrome, chronic kidney disease, fibrosis

## Intellectual Property and Financials

- Patent filed in 2020 on methods of use, formulations and composition of matter
- Seeking \$2M seed funding for operations, scale-up manufacturing, preclinical studies, and pre-IND FDA meeting

# The unmet need: Vascular Leak

## Endothelial Barrier Disorders



Vascular leak cuts across multiple organ systems and disease states

There are more than 60 conditions associated with vascular leak, including:

- ARDS
- Diabetic macular edema
- Acute kidney injury
- Chronic kidney disease
- Acute pancreatitis
- Fibrosis
- Ascites
- Systemic capillary leak syndrome

E. Siddall, M. Khatri, J. Radhakrishnan *Kidney International* (2017)

Vascular leak is an increase in capillary permeability leading to the loss of protein-rich fluid from the intravascular to the interstitial space

# ARDS: a deadly condition with no treatment

Described as “wet lung” in 2014

## Acute Respiratory Distress Syndrome (ARDS)

### 1 Required criteria (must meet all three)

- Timing within 1 week of clinical insult or new/worsening respiratory symptoms
- Chest XR shows bilateral opacities not fully explained by effusions, lobar/lung collapse, or nodules
- Respiratory failure not fully explained by cardiac failure/fluid overload

### 2 Risk factor

- Risk factor for ARDS present (e.g. pneumonia, trauma, sepsis, pancreatitis)
- Objective assessment (Echo) excludes hydrostatic edema if no risk factor present
- None of the above

### 3 Severity (Oxygenation)

- Mild:  $\text{PaO}_2/\text{FiO}_2 >200$  to  $\leq 300$  mmHg with PEEP OR CPAP  $\geq 5$  cm  $\text{H}_2\text{O}$
- Moderate:  $\text{PaO}_2/\text{FiO}_2 >100$  to  $\leq 200$  mmHg with PEEP  $\geq 5$  cm  $\text{H}_2\text{O}$
- Severe:  $\text{PaO}_2/\text{FiO}_2 \leq 100$  mmHg with PEEP  $\geq 5$  cm  $\text{H}_2\text{O}$
- None of the above

NORMAL VERSUS ARDS CHEST SKIAGRAMS :



The reason for mortality  
in COVID-19 pandemic

Before COVID-19

> 400,000 cases  
p.a.

14+ average days  
in ICU

30-40% mortality  
75,000  
deaths/year

**\$1.6B market**

<sup>1</sup>Uckun, F.M., Front.Pharmacol. 2020, 11:796 <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7264370/#B8>

<sup>2</sup>2018 Data, <https://www.openaccessgovernment.org/acute-respiratory-distress-syndrome/43754/>

## Current ARDS standard of care



### News



Classification of  
Omicron (B.1.1.529):  
SARS-CoV-2 Variant of  
Concern

26 November 2021

## Aktyva Therapeutics



- **Mechanical ventilation**
  - Goal: increase oxygenation
- **Problems:**
  - High delivery cost / ICU / skilled nursing
  - Mechanical damage to lung
  - Scarring / fibrosis
  - Palliative (30-40% mortality)

- **Small molecule**
  - Low cost, easy delivery
  - Multiple modes of administration
  - Address the root cause
  - Prevents ICU admission
  - **Virus and mutations-agnostic**

# Aktttyva's Approach to Stop ARDS

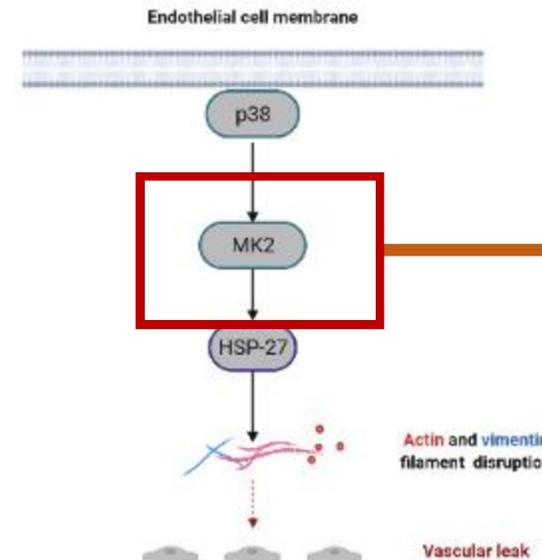
## Targets:

- MK2 pathway involved in stress response when activated
- Pathway activation in endothelial cells stops vascular leak
- *In vivo* proof-of-concept established

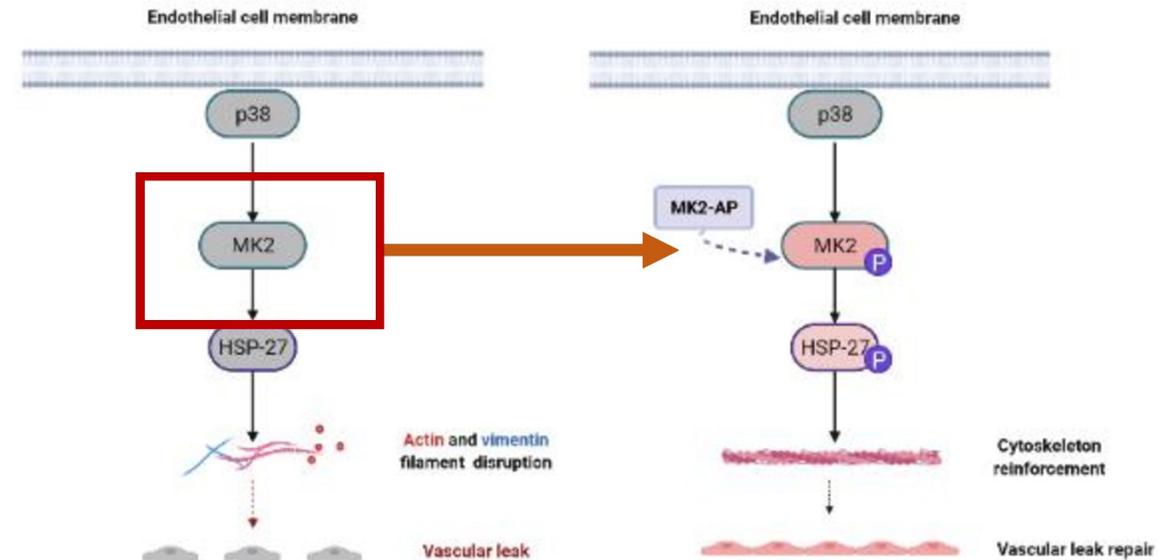
## Approach:

- Direct activation of MK2 pathway with small molecule to stop vascular leak
- **Agnostic to a specific virus, organ or toxic agent**

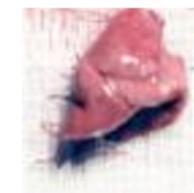
### VASCULAR LEAK



### VASCULAR LEAK REPAIR



**Rat lung edema**



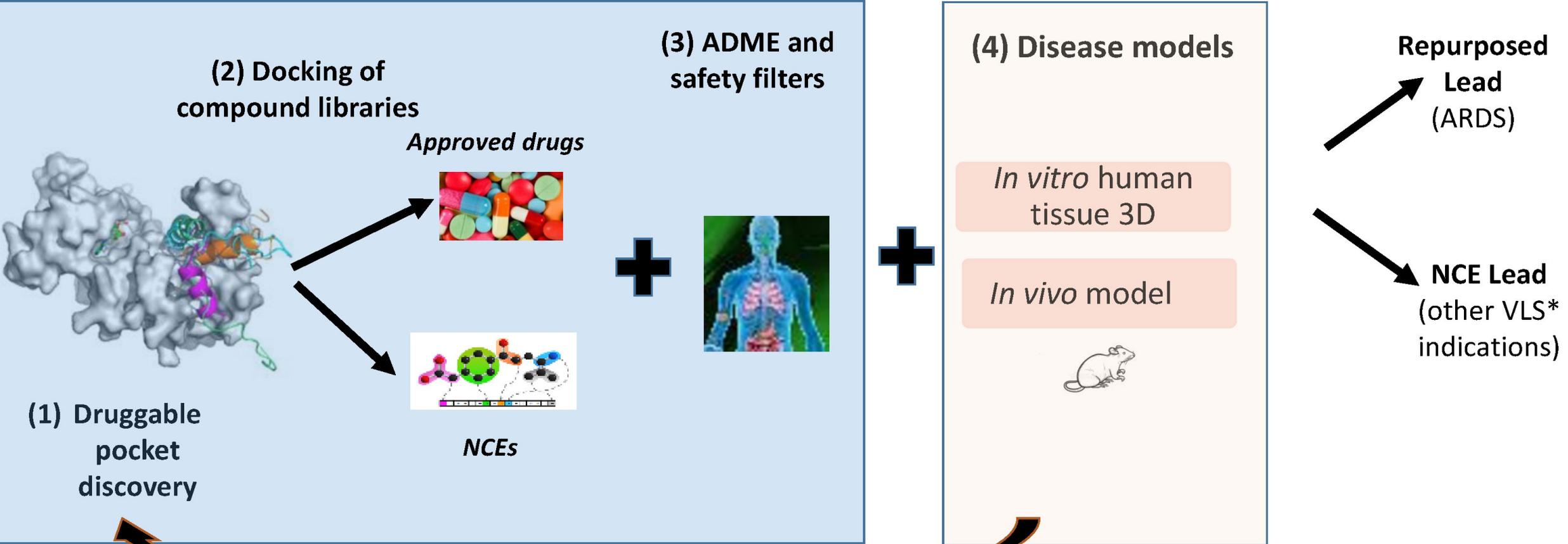
**Rat lung normal**

# Aktyva AI-assisted Drug Discovery Engine

360° *in silico* / *in vitro* analysis



Aktyva's AI-assisted Drug-discovery Engine  
**AIDE<sup>360</sup>**

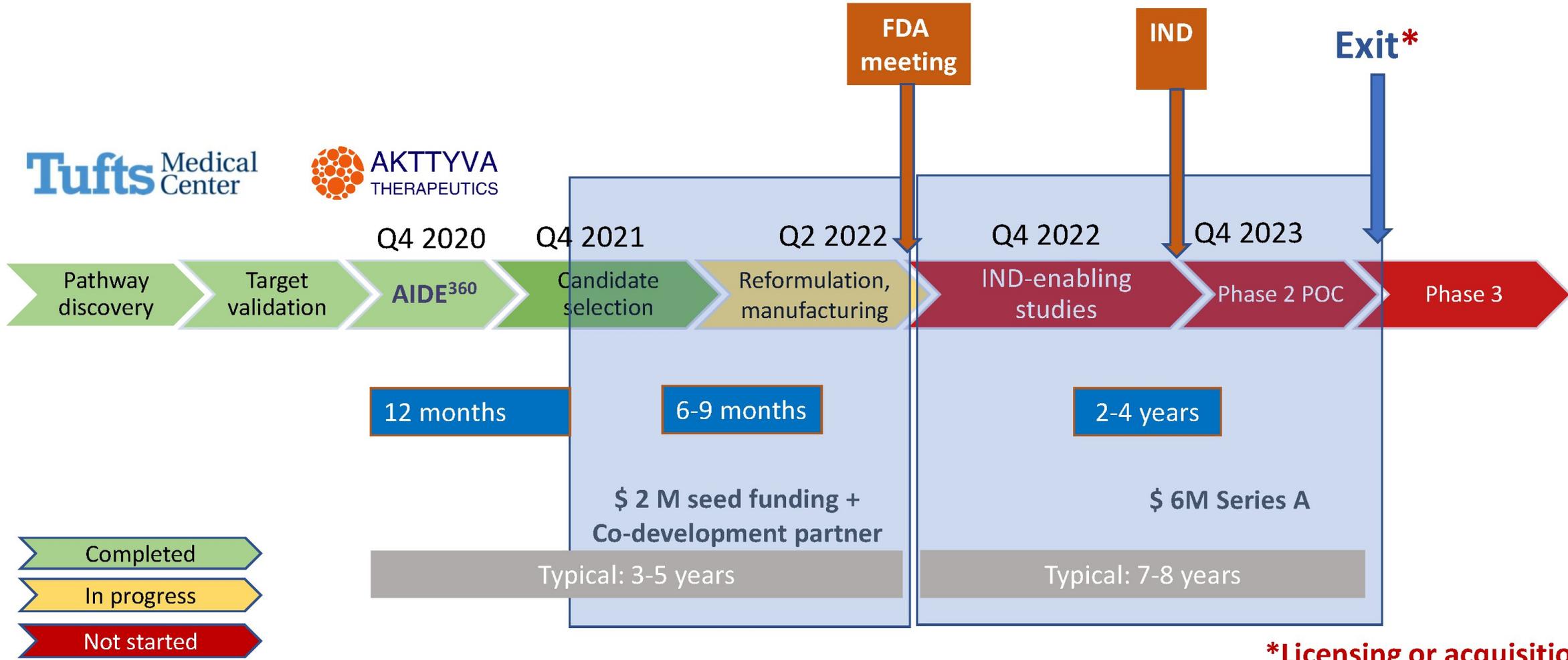


In silico docking optimization

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# Repurposed drug for ARDS treatment

Program designed for efficiency and speed to clinical PoC



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\*Licensing or acquisition

## Use of proceeds:

### Repurposed drugs:

- Lead candidate selection
- Lead candidate reformulation
- Clinical development plan
- Pre-IND meeting with FDA

### New Chemical Entity:

- Use AIDE<sup>360</sup> for NCE discovery for the next vascular leak indication (funded by NSF (+ NIH award pending))

## Current funding:

- ✓ **\$259K** – NSF award 12/1/21
- ❑ **Seed Raise: \$2 M to select lead candidate and advance into clinical development**
  - ❑ **\$200K closed 12/2021**

### **Intellectual Property:**

- Patent application: Compositions of matter, formulations and methods of use covering the small molecules filed in 2020, converted to PCT 11/12/21
- Additional filings planned on NCE modulators of MK2 and other members of the pathway (MK3, MK5)

# Progress and Traction

- ✓ AIDE<sup>360</sup> platform in place:
  - ✓ Utilized for MK2 target:
    - ✓ More than 7,000 existing drugs have been docked to the MK2 pocket
    - ✓ >100 candidate drugs for repurposing identified
    - ✓ Activity assay established in the lab
    - ✓ 5 lead candidates identified based on experimental activity
  - ✓ NCE screening to begin shortly
- ✓ Lead candidate selected for clinical development in ARDS
- ✓ **NSF Award recipient**
- ✓ **Finalist of Mass Challenge 2021**
- ✓ **Award-winner: IBM partnership in AI**



## Why existing drug now?

- ✓ Immediate medical need due to COVID-19
- ✓ Shorter development time to POC in humans
- ✓ Safety profile in humans established
- ✓ Drug supply readily available

# Projected U.S. revenues from ARDS drug



## 1) In-hospital infused

	YEAR 1	YEAR 2	YEAR 3
Patients, US	400,000	400,000	400,000
Penetration rate	10%	20%	30%
Addressable market	40,000	80,000	120,000
ARDS TREATMENT REVENUES (\$)	\$400M	\$800M	\$1,200M
Royalties	\$20M	\$40M	\$60M

### Assumptions:

1. Treatment price of \$10,000 per patient (estimated based on market research by AlientT)
2. Reimbursement:
  1. Medicare Part A

**\* Source:**

Comparables report prepared for Akttyva by AlienTechnologies, Inc., April, 2021

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# Projected U.S. revenues from ARDS drug

## 2) Ambulatory / non-hospital administration



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	YEAR 1	YEAR 2	YEAR 3
Patients, US	400,000	400,000	400,000
Penetration rate	10%	20%	30%
Addressable market	40,000	80,000	120,000
ARDS TREATMENT REVENUES (\$)	\$400M	\$800M	\$1,200M
Royalties	\$20M	\$40M	\$60M

### Assumptions:

1. Projected treatment price of \$10,000 per patient (price per dose TBD)
2. Reimbursement options:
  1. Medicare Part B (physician-administered infusions in hospital outpatient or physician's office)
  2. Medicare Part D (self-administered oral solid or autoinjector)

**\* Source:**

Comparables report prepared for Akttyva by  
AlienTechnologies, Inc., April, 2021

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# ARDS drugs in clinical trials

Company	Drug	Mechanism	Type	Safety	Cost
Faron Pharma	Traumakine (synergies, positive phase 2)	Indirect mechanisms	Expensive complex therapies	Unknown safety	\$\$
BioMarck Pharma	BIO-11006				\$\$
Athersys	MultiStem				\$\$\$
Bayer	BAY1097761				\$\$\$
	BAY 1211163				\$
<b>Akttiva</b>	<b>AKT-001</b>	Targeted therapy	Small molecule	Safety established	\$

Targeted cost-effective therapy focused on patient and human biology

# Team

Co-founded and led 16 companies  
Brought 10 drugs to market  
7 successful exits, 2 IPOs



**CEO,  
Board Director**

**Katya Tsaoun, Ph.D.** – 20+ years in biopharma; successful entrepreneur (founded Apredica, an ADME-Tox CRO in 2006, successful exit to Evotec); expertise in mechanistic strategies of de-risking drug-discovery programs Executive Director of EBTC at Johns Hopkins Bloomberg School of Public Health

Serial entrepreneur,  
De-risking drug candidates

Investor



**Biology**

**Usamah S. Kayyali, Ph.D., MPH, DABT** -Formerly, Associate Professor, Tufts Medical Center, discovered mechanisms of vascular permeability (\$2M NIH-funded research), demonstrated role of p38-MK2-HSP27 pathway in vascular permeability barrier regulation.

Pathway discovery and  
validation



**Head of Research**

**Mario DiPaola, Ph.D.**  in the pharma industry with roles in basic R&D, quality, CMC and regulatory functions in support of protein and small molecule development; regulatory filings (BLA/eCTD) for Alferon-N, Amevive, Sucraid, Authored/co-authored 60+ publications and co-inventor in 4 patents

Assay development,  
biology

Investor



**Chemistry**

**Bruno Villoutreix, Ph.D.** – 25 years interface between molecular medicine / structural-bioinformatics / chemoinformatics, in silico drug design, ADME-Tox, small molecule PPI and protein-membrane modulators, Research Director at Inserm (French medical research Institute)

Biomolecular modeling



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**Board Director**

**Lana Gladstein, J.D.** – Chief Legal Officer, Arranta Bio; previously EVP and General Counsel of Brammer Bio (bought by Thermo Fisher in 2019); formerly partner at Troutman Pepper and partner at Nutter, Boston, MA

Intellectual property



**Board Director**

**Mark Tepper, Ph.D.** Founder and C-level management for Corbus Pharmaceuticals (IPO 2014), Primatope Therapeutics (acquired), NKT Therapeutics, and RXi Pharma (IPO 2007). Previously held positions: CEO, Multiple Life Science startups, VP USA Research & Operations Bristol-Mye



Corporate strategy



# Advisors



Translational, M&A  
expertise

## Vanessa Carle, Ph.D.

Associate, **Pharma Ventures**  
Expertise in drug discovery R&D,  
technology commercialization,  
translational drug-development projects  
and the start-up ecosystem, out-licensing  
partnerships. **Winner of MassChallenge  
Switzerland.**

**PharmaVentures**  
— the deal experts —



Drug reimbursement  
strategy

## David Farber, J.D.

Partner, **King & Spaulding**, FDA &  
Life Sciences Practice  
Litigation, regulatory matters, and  
public policy, with a focus on  
healthcare, Medicare and Medicaid  
issues for pharmaceutical and medical  
device companies, hospitals and  
pharmacies. He is a recognized  
national expert on Medicare  
Secondary Payer issues.



Critical care physician

## Prof. Hugh Montgomery, MB BS BSc FRCP MD FRGS FRI FFICM

Professor of Intensive Care  
Medicine, University College  
London  
Conducted numerous clinical  
trials in pulmonary indications  
including ARDS indication

### BRIGHAM HEALTH



CMC, Regulatory  
strategy

## Steve Kates, Ph.D.

Drug development executive with ~30 years'  
experience in the drug development in infectious,  
cardiovascular, renal and CNS diseases. CMC (Drug  
Substance and Drug Product manufacturing),  
regulatory and medical affairs. Writing and review of  
scientific and regulatory documentation (INDs, NDAs,  
BLAs, ANDAs, eCTD).

**Focus:** addressing unmet medical need

Proprietary efficient screening platform

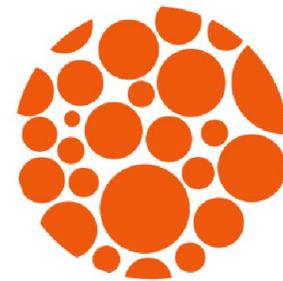
Advanced ARDS program ready for IND-enabling studies

Platform to address a key pathology in > 60 indications

**De-risked by** NSF, angel investors funding of \$430,000

Thank you for your attention

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**AKTTYVA**  
**THERAPEUTICS**

Katya Tsaïoun, Ph.D.  
Founder and CEO  
[www.Aktyva.com](http://www.Aktyva.com)  
[Katya@Aktyva.com](mailto:Katya@Aktyva.com)  
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