

# In the Global Fight Against Infection

March 2021

TSX: APLI / OTCQX: APLIF



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# APPILI THERAPEUTICS: TACKLING TODAY'S GLOBAL ID CHALLENGES



## ID Portfolio Company

Built to identify and advance promising infectious disease (ID) programs



## Founded by Investment Bank

Backed by leading investment advisory firm



bloom burton & co



## Diversified Pipeline

Diversified pipeline of 5 programs with multiple near-term milestones



## Skilled Leadership Team

Leadership team skilled in advancing programs with upside potential

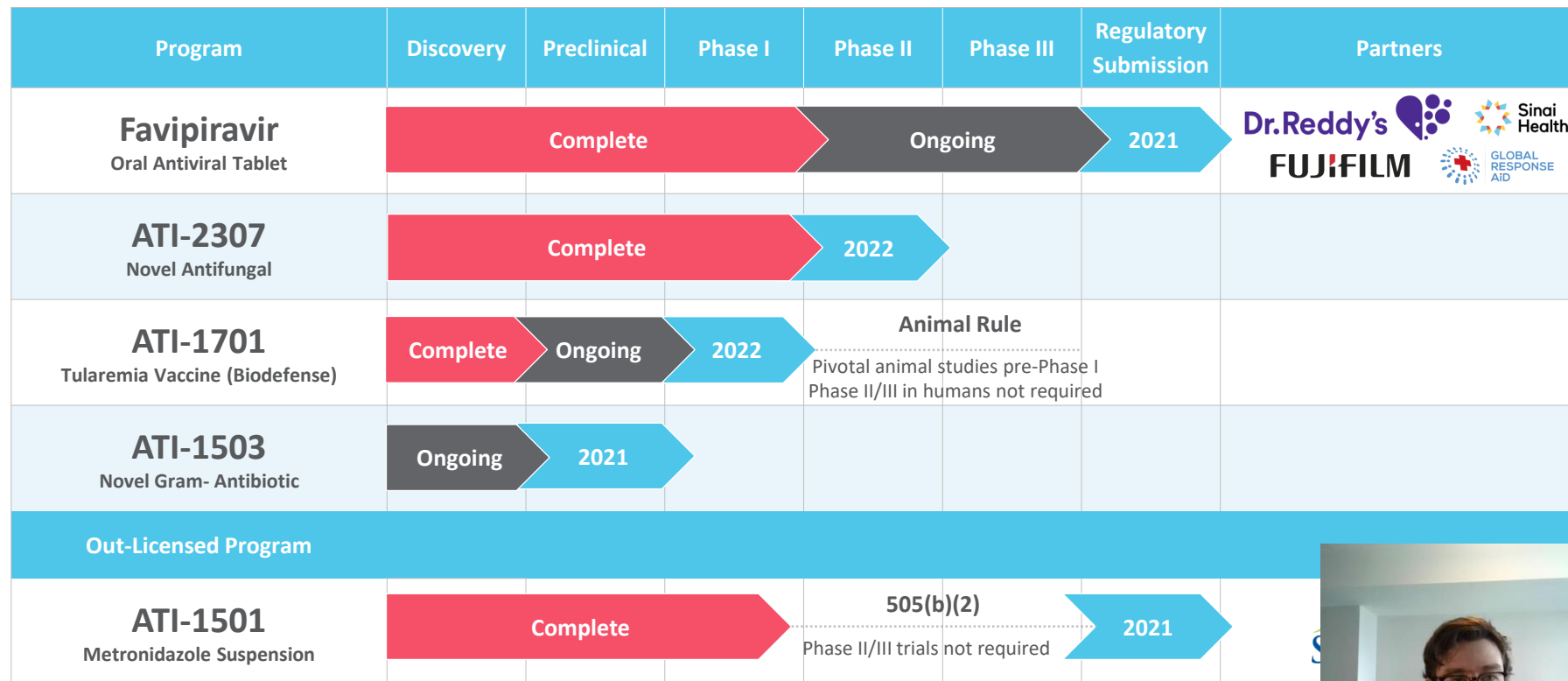


## Business Development

Active in-licensing / partnering to expand clinical programs and accelerate commercialization



# DIVERSIFIED PIPELINE DRIVING NEAR- AND LONG-TERM VALUE



 Complete
  In Progress
  Planned




























# Favipiravir

Antiviral for Treatment and Prevention of COVID-19



# THE COVID-19 TREATMENT SPECTRUM

	No Therapy	Vaccine (Not Approved)	Avigan® (favipiravir)	Other R&D Antivirals (Not Approved)	Monoclonal Antibodies (EUA)	Convalescent plasma (EUA)	Remdesivir (Approved)	Dexamethasone (Not Approved)	Ventilators & Respiratory Support
 Pre Exposure No Disease									
 Post Exposure Unknown Disease									
 Asymptomatic									
 Mild Disease									
 Moderate Disease									
 Severe Disease									

 Approved Treatments at this stage



# FAVIPIRAVIR OVERVIEW



**APPILI**  
THERAPEUTICS

**Dr.Reddy's**



GLOBAL  
RESPONSE  
Aid

**FUJIFILM**

## Favipiravir





- Novel, broad-spectrum oral antiviral
- Approved in Russia and India for COVID-19
- Well-understood mechanism targeting essential viral polymerase
- Promising trial data for mild to moderate COVID-19
- Extensive clinical experience, safety database > 3,000 subjects
- Oral tablet suitable for use at home and outside the hospital
- Shelf stable and compatible with existing distribution channels

## Global coalition advancing favipiravir for COVID-19

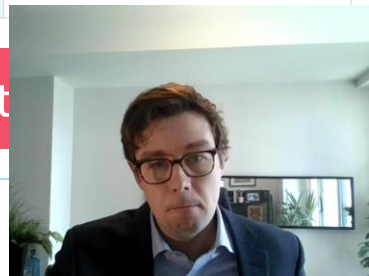
- Appili working with global partners FUJIFILM, Dr. Reddy's, Global Response Aid
- Team built to support rapid development, and sustainable long-term supply
- Clinical program underway to definitively assess safety and
- Engaging with governments on access / supply



# AVIGAN® (FAVIPIRAVIR) ORAL ANTIVIRAL CLINICAL PROGRAM

Program	Discovery	Preclinical	Phase I	Phase II	Phase III	Regulatory Submission	Partners
<b>Avigan® (favipiravir)</b> Oral Antiviral Tablet	Complete		Ongoing		2021		   
<b>PRESECO</b> Early Treatment Community Ph III US + LATAM / N > 800				2020 / 2021			
<b>PEPCO</b> Post-Exposure Prophylaxis Community Ph III US + Canada / N > 1,100				2020 / 2021			
<b>CONTROL</b> Long-Term Care Outbreak Control Ph II Canada / N > 700			2020 / 2021				

Focused on early-stage community infections in high-risk patients





# UPDATE ON APPILI TRIALS



**Now enrolling in two out of three planned trials including pivotal treatment Phase 3 PRESECO**

- Overall program focused on early administration to limit spread and severity of COVID-19 in at-risk groups
- Trials include Phase 2 in Ontario long-term care and two Phase 3 early treatment and prophylaxis studies
- Phase 2 enrolling in Canada, treatment Phase 3 now enrolling, and prophylaxis Phase 3 under review
- Integrated with global partners to support global regulatory approvals including Canada and US



**Robust trials focused on early intervention for patient groups at highest risk for severe COVID-19**

## Phase 3

**Early Treatment  
COVID-19 Trial  
PRESECO**

*Status: **Enrolling***  
*Interim Look: **Q2 2021***  
*Top-Line: **Q2 2021***

## Phase 2

**COVID-19 Outbreak Control  
in Ontario Long-Term Care  
CONTROL**

*Status: **Enrolling***  
*Top-Line: **Q4 2021***

## Phase 3

**COVID-19 Post-Exposure  
Prophylaxis Trial  
PEPCO**

*Status: **Enrolling***  
*Top-Line: **Q4 2021***



# GLOBAL CONSORTIUM TO DEVELOP ORAL ANTIVIRAL FAVIPIRAVIR



## Global coalition advancing favipiravir for COVID-19

- Appili working with global partners to develop leading COVID-19 antiviral candidate favipiravir
- Consortium includes innovator FUJIFILM, Dr. Reddy's Laboratories, and Global Response Aid
- Team built to support rapid development, regulatory submissions, and sustainable long-term supply
- Clinical program underway to definitively assess favipiravir's role in COVID-19 care



GLOBAL  
RESPONSE  
Aid

## Coalition Advantages

- ✓ **Proprietary Data:** Access to FUJIFILM data and IP accelerate development and block competitor entry
- ✓ **Robust Trial Infrastructure:** Coordinated global program with multiple near-term trial readouts
- ✓ **Global Manufacturing Scale and Reach:** Leading global manufacturing and distribution network to maximize supply and revenue for patients



# THE VALUE OF ORAL ANTIVIRALS



## Benefits of oral antivirals

- Opportunity to rapidly intervene prior to or early after infection when viral loads are highest
- Potential to limit spread of disease, duration and progression to severe illness, hospitalizations
- Significant public health and economic benefits enabling reopening of the economy
- Durable need even after vaccine is available, protecting and treating high-risk groups including the elderly



Prior experience with Roche's Tamiflu® underscores value of oral antiviral, even when vaccine is available

### 2009 H1N1 Influenza Pandemic

**Over \$3B**

*Global Tamiflu® Sales  
2009*

**8.7M / \$905M**


*Estimated US Outpatient Rx / Sales  
Excluding Stockpile  
2009*

### Seasonal Influenza

**Over \$500M**

*Annual US Sales  
2009-2014*





**ATI-2307**

Novel Clinical Stage Antifungal



# URGENT UNMET NEEDS IN *CRYPTOCOCCUS* AND *CANDIDA*

## *Cryptococcus*

- Opportunistic, invasive infection causing meningitis
- Heavy disease burden globally with high mortality
- Suboptimal outcomes with toxic standard of care

### Global

- Neglected, decades old public health crisis
- Unacceptable loss of life, political will to fix

### US+

- Severe infections treated with toxic agents
  - In-hospital mortality over 10%
  - Average hospital stay 15 days
  - Costs estimated over \$70K / case
- Underserved and growing orphan segment

## *Candida*

- Among the most common fungal pathogens
- Resistance threatening existing antifungal arsenal
- CDC estimate **over 34K** drug-resistant cases in US annually
- Last resort agent amphotericin B is highly toxic
- Multiple segments of urgent unmet need, including:
  - Refractory and resistant *Candida* UTI
  - Emergent, highly resistant *C. auris* infections

**The New York Times**  
April 6, 2019  
***A Mysterious Infection, Spanning the Globe in a Climate of Secrecy***

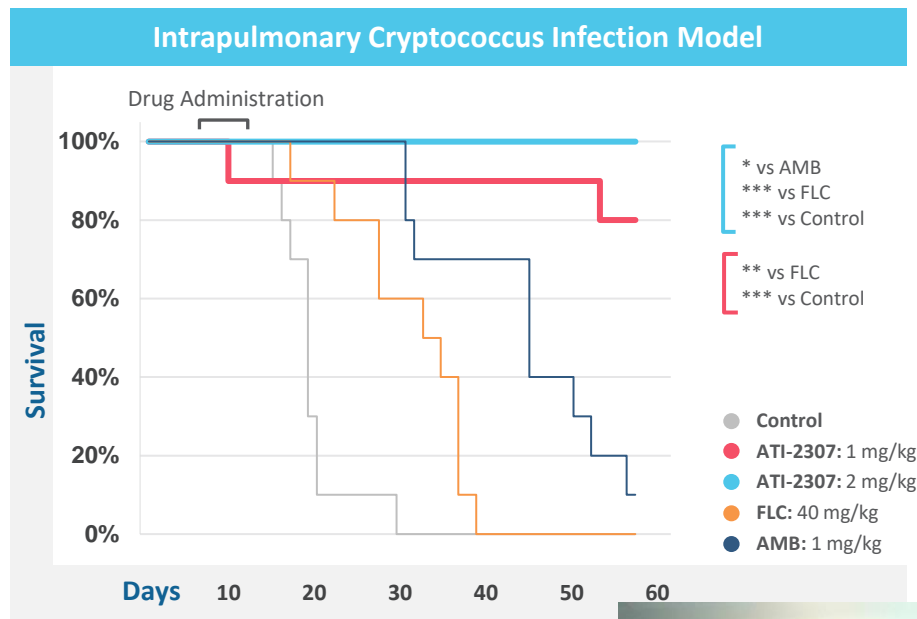
The rise of *Candida auris*  
public health threat: drug



# ATI-2307: A NOVEL CLINICAL STAGE ANTIFUNGAL

- A novel antifungal with broad spectrum activity against a wide array of fungi, including *Candida*, *Aspergillus* and *Cryptococcus*
- 100% survival in lethal lung infection model
- Evaluated in 3 Phase 1 studies; safe and well tolerated at anticipated Phase 2 dose levels

Survival Data  
AMB = Amphotericin B  
FLC = Fluconazole  
\* / \*\* / \*\*\* =  $p < 0.05$  /  $0.01$  /  $0.001$  by log-rank test



New option for physicians to overcome difficult to treat and resistant fungal infections like Crypto



# MULTIPLE ATTRACTIVE MARKET OPPORTUNITIES

## US Orphan Cryptococcal Meningitis Market

**Over 5,000 Rx**

Estimated amphotericin B Rx / year for CM and cryptococcosis

 + Appili Analyses

**\$60K-\$90K per Rx**

Premium pricing supported by payer research\*

 **RESEARCH AMERICA**  
MARKET RESEARCH · CONSUMER INSIGHT

**Over \$350M market opportunity**

Annual US market potential at \$70K per Rx

## + US Refractory / Resistant *Candida* Markets



2019 AR  
Threats Report

**Drug-Resistant *Candida***

**34.8K Cases / 1.7K Deaths**

Estimates for 2017

***Candida auris***

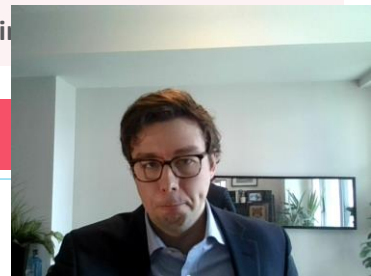
**90%** resistant to at least ONE antifungal

**30%** resistant to at least TWO antifungals

**318%**

Case i

## + Ex-US Markets



# ANTIFUNGAL BENCHMARKS AND VALUATIONS

## Recent Approval: Cresemba®



- Developed by Basilea
- Azole Derivative
- FDA Approval in 2015

## US License

**Total Value: Over \$400M**

+ double-digit royalties



Signed in 2010, amended in 2014, 2015

## EU + APAC License

**Total Value: Over \$700M**

+ mid-teen royalties



Signed and amended in 2017

## Valuations & Funds Raised\*

### Phase 2



**\$90M+ Series C**

2017-2020



**\$120M Raise**

2016 & 2019

### Phase 3



**\$145.1M**

NASDAQ: SCYX, Feb 1, 2021



**\$113.8M**

NASDAQ: CDTX, Feb 1, 2021

+ Ex-US/Japan Partnership

**Total value: Over \$568M**

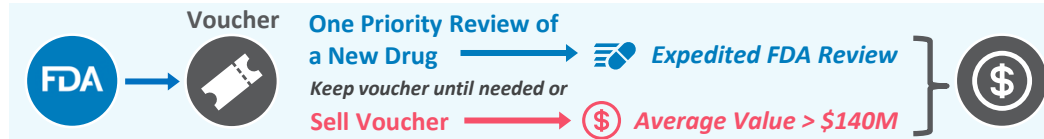
+ double-digit royalties





# PRIORITY REVIEW VOUCHER ELIGIBLE

If approved by the FDA for cryptococcal meningitis, candidate would be eligible for a priority review voucher



## What is a priority review voucher (PRV)?

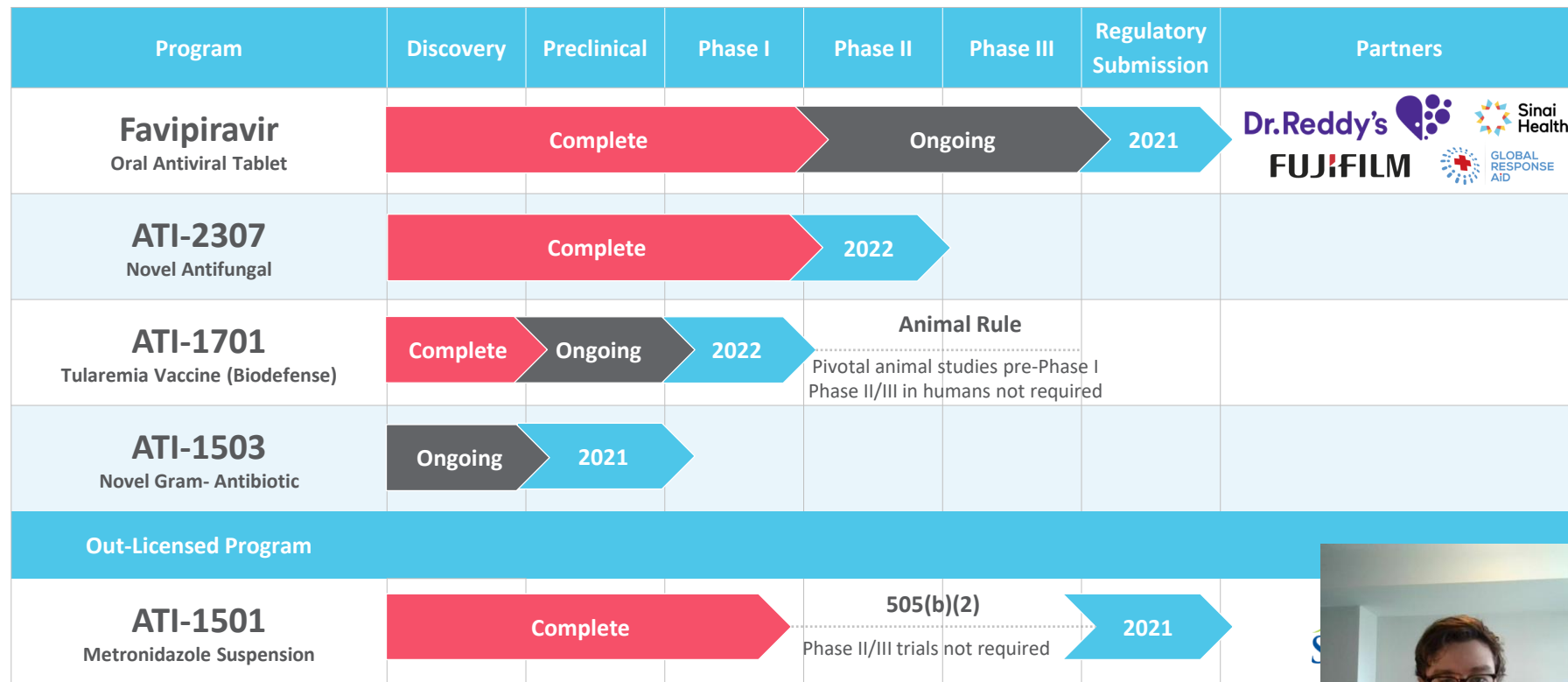
- Allow holder to accelerate FDA review of any NDA
- Granted by FDA to reward R&D in target areas
  - Rare pediatric disease
  - Tropical disease
  - Biodefense
- PRVs are **transferrable**, average price >\$140M

Date	Sell	Purchaser	Value (USD)
H2 2014	BioMarin	Sanofi / Regeneron	\$67.5M
H2 2014	Knight Therapeutics	Gilead	\$125M
H1 2015	Asklepion / Retrophin	Sanofi	\$245M
H2 2015	United Therapeutics	AbbVie	\$350M
H2 2016	PaxVax	Gilead	\$290M
2017	5 separate transactions		\$125M - \$150M
H1 2018	Spark Therapeutics	Jazz Therapeutics	\$110M
H1 2018	Ultragenyx / Kyowa Hakko Kirin	Gilead	\$80.6M
H2 2018	SIGA Technologies	Eli Lilly	\$80M
H1 2019	GW Pharmaceuticals	Biohaven	\$105M
H2 2019	SOBI	AstraZeneca	
H2 2019	Bavarian Nordic	-	
H1 2020	Sarepta Therapeutics	Vifor Pharma	
H2 2020	Lumos Pharma	Merck	

At least two additional transactions with no financials disclosed, both involving Novo Nordisk  
 Primary reference GAO-20-251, with supporting data from press releases or financial disclosures



# DIVERSIFIED PIPELINE DRIVING NEAR- AND LONG-TERM VALUE



 Complete
  In Progress
  Planned



# BUSINESS DEVELOPMENT: BUILDING AND ADVANCING ID PIPELINE



Company built to find and advance ID programs



Robust in-licensing strategy to identify overlooked assets

- Agnostic to any particular platform or technology
- Pharma, academia, government agencies
- Constantly analyzing programs to identify those that can address compelling unmet needs



Establishing relationships with pharma for future commercialization



# SKILLED MANAGEMENT TEAM



**ARMAND BALBONI**

*CHIEF EXECUTIVE OFFICER*

Extensive drug development experience in civilian, academic, and military organizations



**MYRIAM TRIEST**

*SR. DIRECTOR, MANUFACTURING AND PHARMACEUTICAL DEVELOPMENT*

15+ years as a drug development professional and PhD chemist; discovery to Phase 3



**YOAV GOLAN, MD**

*CHIEF MEDICAL OFFICER*

30+ years as an infectious disease physician; published research on *C. difficile* infections and invasive candidiasis



**STÉPHANE PAQUETTE**

*SENIOR DIRECTOR, CORPORATE DEVELOPMENT*

10+ years infectious disease and industry R&D experience; PhD in virology & immunology



**KIMBERLY STEPHENS**

*CHIEF FINANCIAL OFFICER*

CPA, CA 18+ years of financial management and public company experience



**JASON MCEWAN**

*DIRECTOR, REGULATORY AFFAIRS*

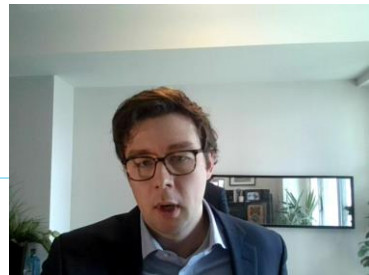
15+ years of regulatory consulting for Canada and the US; part of team receiving the Deputy Minister Award for his work during a major global healthcare crisis.



**DON CILLA, PHARMD, MBA**

*CHIEF DEVELOPMENT OFFICER*

30+ years of program management experience in the drug development industry



# BOARD OF DIRECTORS



**IAN MORTIMER**

*CHAIR*

President and Chief Financial Officer of Xenon Pharmaceuticals Inc, 20+ years of experience in the biotechnology sector



**BRIAN BLOOM**

*MEMBER*

Chairman and CEO of Bloom Burton & Co, 18+ years of capital market experience



**JUERGEN FROEHLICH, MD**

*MEMBER*

30+ years of biotech experience including all phases of drug development and regulatory interactions



**THERESA MATKOVITS, PhD**

*MEMBER*

20+ years of experience as a leader in global drug development, with extensive expertise in infectious disease



**ROCHELLE STENZLER**

*MEMBER*

25+ years of experience as a board director and senior operating executive in healthcare and other industries.



**ARMAND BALBONI**

*MEMBER*

Extensive drug development experience in civilian, academic, and military organizations



# FINANCIAL OVERVIEW AND CAPITAL STRUCTURE

## FINANCING (As of February 15, 2020)

### Capital Raised:

- **\$68M** raised in total
  - **\$43.6M** in equity
  - **\$25.3M** in government assistance

### Cash & cash resources (September 30, 2020)

- **Cash & Short-term Investments:** **\$22.9M**
- **Government grants over 2.5 years:** **\$2.6M USD**

## CAPITAL STRUCTURE (As of February 15, 2020)

**62.4M** Common shares outstanding

**14.9M** Warrants

**4.5M** Options

**81.8M** Fully diluted

## STOCK INFORMATION (As of February 15, 2020)

**TSX: APLI**      Graduated to TSX September 16, 2020  
**\$0.50 - \$1.89**      52 week low-high  
**\$75M**      Market Cap

## SIGNIFICANT OWNERSHIP

Bloom Burton & Co.  
K2 Principal Fund L.P.  
Innovacorp



# NEWS FLOW AND MILESTONES

Milestone	Program	Expected Date	Completed
Prospectus Financing	Corporate	Q1-2020	✓
Regulatory clearance for COVID-19 Clinical Trial(s)	Favipiravir	Q2 2020	✓
Graduation to the TSX	Corporate	Q3-2020	✓
First enrollment for COVID-19 Clinical Trial(s)	Favipiravir	Q4-2020	✓
Partnership for a New Asset / Indication	Corporate	Q4-2020	✓
Bolstering Industry Focused Board	Corporate	Q1-2021	✓
Interim Data readout (PRESECO Trial)	Favipiravir	Q2-2021	
Full data readout(s)	Favipiravir	Q2-2021	
NDA Submission	Favipiravir	H2-2021	
<i>Cryptococcus</i> Proof of Concept Nonclinical Results	Antifungal (ATI-2307)	H2-2021	
Phase 2 Clinical Study Start	Antifungal (ATI-2307)	H1-2022	



# CONTACT

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