

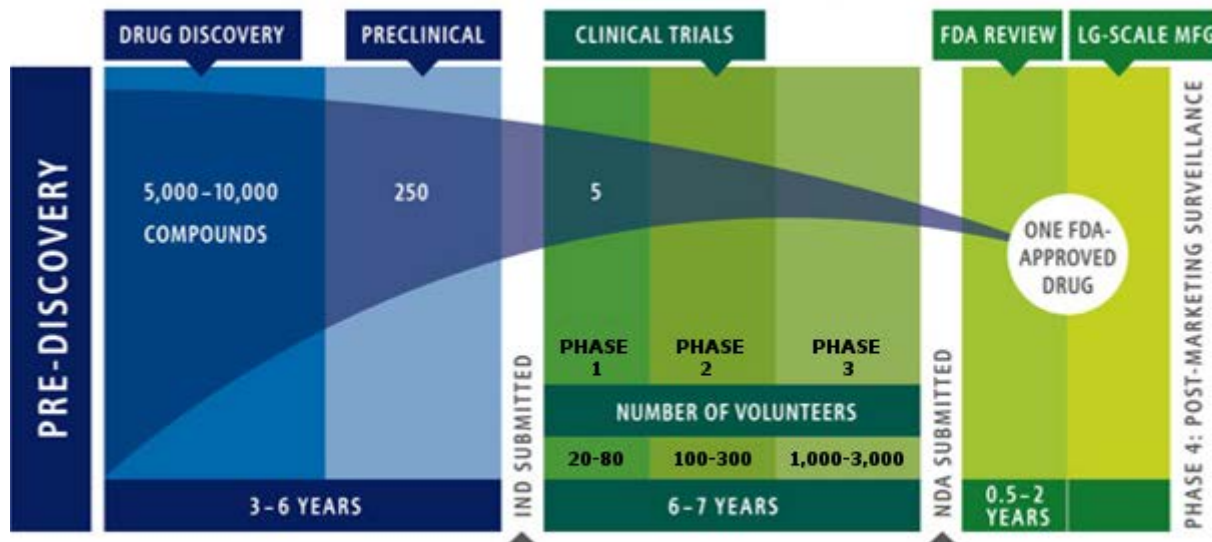
# Realities and Challenges of Pharmaceutical Development

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# We have all heard the statistics...

A recent research by Small Biz Trends shows that about 90% of the new startups fail.

**90%**



Source: Pharmaceutical Research and Manufacturers of America

**Table 1: Probabilities of Phase Failure by Disease Group**

The table shows the average probability of failing each phase of the FDA drug development process, broken down by disease groups. These failure rates are from data from 2006-2015, and are taken from Thomas et. al. (2016).

Disease Group	Probability of Failing Phase Conditional on Reaching It			NDA/BLA Approval Phase	Overall Probability of Failure
	Phase 1	Phase 2	Phase 3		
Hematology	27%	43%	25%	16%	74%
Infectious Disease	31%	57%	27%	11%	81%
Ophthalmology	15%	55%	42%	23%	83%
Other Disease Groups	33%	60%	30%	12%	84%
Metabolic	39%	55%	29%	22%	85%
Gastroenterology	24%	64%	39%	8%	85%
Allergy	32%	68%	29%	6%	85%
Endocrine	41%	60%	35%	14%	87%
Respiratory	35%	71%	29%	5%	87%
Urology	43%	67%	29%	14%	89%
Autoimmune/immunology	34%	68%	38%	14%	89%
Neurology	41%	70%	43%	17%	92%
Cardiovascular	41%	76%	45%	16%	93%
Psychiatry	46%	76%	44%	12%	94%
Oncology	37%	75%	60%	18%	95%

# What is it REALLY like to be the little guy?

## The good

- Pivot, move quickly, innovation encouraged
- State economic incentives
- Cheaper
- Virtual labs are possible

## The bad

- Isolated
- Inexperienced investors

## The ugly

- Funding (equity or grant). Lack thereof or just plain slow.
- Inexperienced management
- Lack of industry guidance (or even interaction)
- Discrimination

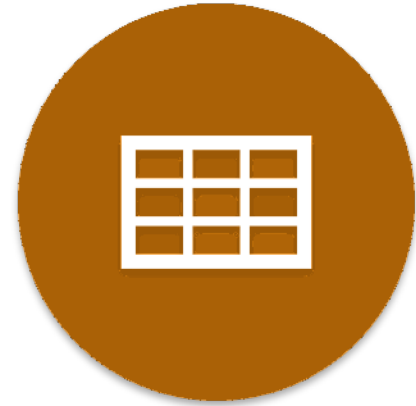
# Time is everything



FUNDING  
TIMELINES



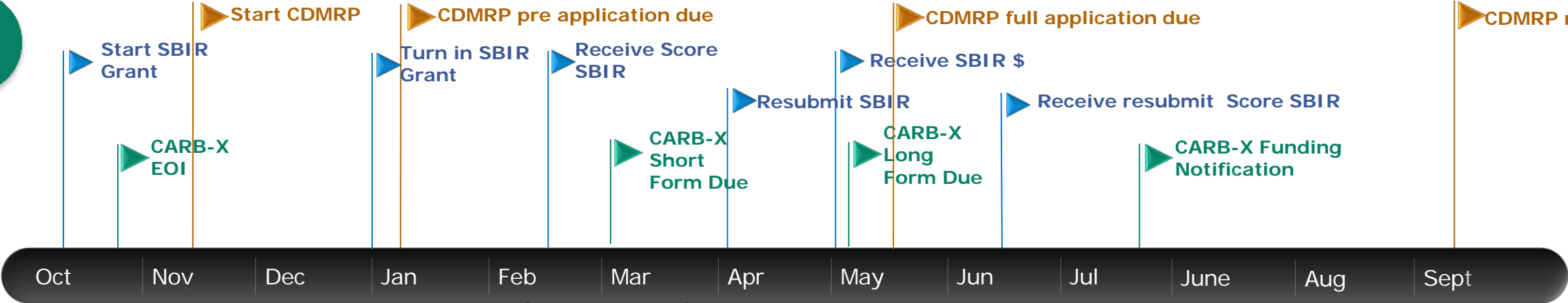
EXPERIMENTAL  
TIMELINES



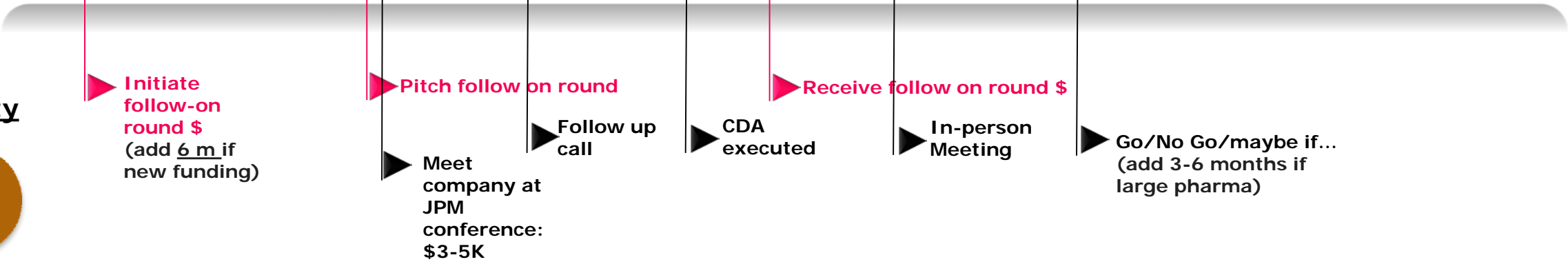
DEAL TIMELINES

# Reality of timing for funding

## Grants



## Equity



\* Assuming no government shutdown



# Experimental timing: Example of Animal experiment

## In-house chemistry, vivarium: 5 weeks

- 1 day of planning
- 1 week to get protocol approved
- 1 week to get animals
- 1 week to acclimate
- 1 week to do the experiment
- 1 week analysis

- **Outsourcing animal work (small company)?**  
Add 2-4 weeks
- **Outsourcing animal work (large company)?**  
Add 3-6 weeks  
(horror story: 7 months!)
- **Outsourcing chemistry?**  
Add 1 month

# Synereca Pharmaceuticals

## Great early stage data and momentum

- Potent, low toxicity
- Experienced management, great advisors
- Finalist for CARB-X, high scores on CDMRP grant
- Capital from local investors

## However...

- Too early for startup
- Funding purgatory (too early, too late)
- Differing feedback (“if you just did...”)

# Pamlico BioPharma

## Good early stage data

- Highly specific, low chance for resistance
- Experienced management
- Capital from local investors
- Sublicense with diagnostic company

## However...

- Too specific
- Challenging patent protection
- The business case was too hard
- Science and the COGS were challenging

# Are these challenges all startups have?

## Yes, to some degree

- Money, inexperience, and resources are always a challenge

## However:

- Type of market and potential ROI helps
- Other startup companies:

**Diabetes Company-** Clear industry milestones. Big market. Grant funded. Failed but failed fast with guidance from potential partners.

**Hearing Loss Company-** High market interest, orphan opportunities, not very crowded. Grant funded, investor funded, multiple deals on the table.

**NASH Company-** High market interest, clear interest and milestones from potential partners and investors.



# Changing the Outlook



## Earlier help from pharma

- Need large pharma to invest, guide, mentor
- How do we incentivize?



## New business/partner models

- Accelerators, collaborative research agreements, industry-academia-startup advisory committees



## Experience

- Startups are usually young, hungry innovators...but they need guidance on company management, drug development, deal making, etc.



## Support

- For younger entrepreneurs (and minorities and women)
- For early stage innovation

# Questions?

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