Molecular Glues as Highly Potent and Targeted Bifunctional Degraders for Undruggable Target Proteins

# Molecular Glues as Highly Potent and Targeted Bifunctional Degraders for Undruggable Target **Proteins**

**COMPANY NAME** 

XXX

YEAR FOUNDED

2015

**INVESTMENT STAGE** 

**EMPLOYEES** 

2-10

**TECHNOLOGY** 

**HEADQUARTERS** 

**USA** 

**TOTAL FUNDING** 

TYPE OF OWNERSHIP

**Public company** 

**KEY MARKETS** 

**TOTAL REVENUE** 

\$31.1 M (2022)

#### ABOUT THE COMPANY

XXX is a clinical-stage company that develops early-stage therapies to transform the treatment of diseases, such as cancer, by targeting disease-causing proteins through its discovery and development platform that enables the development of protein degrader molecules by leveraging the body's natural protein recycling system. The company's approach uses cells' inbuilt machinery to target diseases and potentially provide patients with profound and long-lasting responses.

**KEY CONTACT** 



**NAME** 



**PHONE** 



**EMAIL** 



# **Technology Snapshot**

The company has developed the C4T TORPEDO (Target ORiented ProtEin Degrader Optimizer) platform to synthesize small degrader molecules to selectively and efficiently target disease-associated proteins and degrade them, including previously undruggable targets. The C4T platform encompasses an integrated quantitative design that helps in synthesis, leveraging computational models and aggregated data on numerous highquality degrader molecules degrading target proteins, combining with XXX' expertise in the structural biology of E3 ligases and computational models to better understand the interactions of the ternary complex of the E3 ligase, degrader molecule, and targeted protein. This understanding powers the C4T TORPEDO platform chemistry engine, allowing the company to improve the precision and effectiveness of degrader molecules.

# What Problem does the thechology solve?

Traditional small molecules face limitations in protein inhibition because of a lack of accessible active binding sites, which makes only about 15% of proteins druggable by small-molecule inhibitors. XXX's therapeutic approach to overcome this challenge is targeted protein degradation, which allows access to a significant proportion of possible target proteins that are undruggable with existing strategies. Degraders can bind to any site on the targeted protein to form a ternary complex and initiate the degradation of the target protein, unlike inhibitors, which require an active site to bind and act.

#### **Attributes**

#### **High Efficacy**

The platform can analyze, design, and evaluate the performance of degraders, making drug discovery efficient by enabling quick delivery of effective drug candidates and high in vivo efficacy.

#### **High Catalytic Efficiency**

The platform focuses on improving catalytic activity, which enables the design of degraders in a way to maximize the overall degradation and boosts efficacy.

#### Focus on Cereblon

The TORPEDO platform relies heavily on cereblon, offering numerous target selection options. The attractive features of cereblon binders benefit the company's programs.

# **XXX- Value Proposition**

#### **Technology Assessment**

XXX is developing degraders that can degrade multiple disease-causing proteins with a single degrader molecule through proteasome degradation, unlike its competitors, where a single inhibitor drug molecule can target a single disease-associated protein at a time. Cereblon, the only clinically validated ligase for targeted protein degradation, powers the C4T platform. Cereblon is an appealing target for immunomodulatory drugs, particularly in oncology, and is present in all tissues and cells. The company's distinct IP on cereblon binders increases oral bioavailability, solubility, stability, and permeability during the drug discovery process. Its product candidate has the potential to overcome the limitations of approved IKZF1/3 degraders for multiple myeloma, as C4's products use catalytic activity that allows effective and deep target degradation. The candidate's selectivity reduces off-target reactions, in contrast to off-target toxicities relating to IKZF1/3 degraders. Additionally, the candidate's high binding affinity will prevent resistance development, as with IKZF1/3 degraders.

## **Key Competitors**

- XXX
- XXX
- XXX
- XXX

# **Strategic Analysis**

Opportunities

degradation activating compounds (MonoDACs) that bind to the E3 ligase to increase its affinity for binding target proteins and bifunctional DACs (BiDACs), which have 2 ends to bind to target proteins and E3 ligases, providing targeting flexibility.



 The company's pipeline is in the early developmental or discovery phase. It has yet to determine the technology's efficacy and other vital endpoints.

# **IP/ Patent Activity**

- The company has 27 granted patents and 144 filed patents valid across the United States, China, Brazil, and Argentina.
- Some notable patents include:
  - XXX
  - XXX

 XXX' technology platform is applicable to various disease areas, such as neurological diseases and senescence.

• The platform can develop 2 types of

degraders, including monofunctional



 Competition from other companies working on targeted protein degradation could pose a threat to the company.

#### **Future Focus Areas**

- The company is planning an IND submission for CFT8919, an EGFR L858R BiDAC Degrader, in the next few months and expects CFT8919 to be in clinics by the end of 2023.
- It plans to advance the dose escalation portion of CFT-1946 in Phases 1 and 2.
- The company may enter into new collaborations to explore the applications of its TORPEDO platform.

# **Risk Analysis**



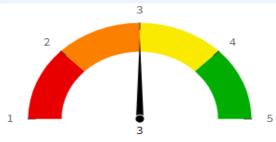
- The company has reported positive initial outcomes for CFT8634, an oral BRD9 degrader, which showed dose-proportional exposure, strong oral bioavailability, and intense BRD9 degradation.
- XXX is the only company developing MonoDAC and BiDAC degraders.

#### **Revenue Magnitude Potential**



• XXX' pipeline is in an early stage of development, though it is the only company developing BiDACs. Its revenue potential should be medium to high in the long term.

#### **Buzz Worthiness**



• The company presented a preclinical evaluation of CFT1946 to the American Association for Cancer Research in 2022 and a paper on the discovery and characterization of its CFT7455 and CFT8634 product candidates.

#### **Investor Lens**



• In 2019, the company partnered with Biogen to research and develop targeted protein therapies for neurological conditions, from which XXX is eligible to receive \$415 million.

# **XXX- Investor Dashboard**

# -NNOVATION IN

D

### **Commercialization Readiness Level**



 The company's pipeline is in an early stage of development, with most of its candidates in the discovery or preclinical phase. XXX is working on other partnered programs, its oncology pipeline, and other disease indications, such as neurodegenerative conditions, including Alzheimer's and Parkinson's.

#### **Technology Competition Level**



 Competition exists from other targeted protein degraders, such as IKZF1/3 and PROTAC protein degraders. Other molecular glue and multivalent degraders are under development.

# **Regional Impact**



• The technology will impact the US and European market.

#### **Application Potential**



- Oncology
- Neurological diseases
- Senescense

# **Analyst's Insights**

XXX developed the TORPEDO platform, which led to the development of molecular glues and bivalent degraders to achieve targeted protein degradation. The company's technology has the potential to transform disease treatments of diseases with undruggable targets. XXX may seek new partnerships to increase the platform's applicability to various disease indications.