Needs Assessment Report*

Company Information

Company Name:	Company ABC
NIH Project Number:	R43AR873964-01A1
Funding Institute/Center:	National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)
Company Contact:	Company ABC Representative

Assessment Information

Assessment Lead:	RTI Innovation Advisor Assessment Lead
Report Date:	June 2, 2022

* This needs assessment report was developed to serve as a sample needs assessment work product. All names of companies, project numbers, products, technologies, therapies, and/or scenarios are fictional.

Product or Service Summary

Company ABC has developed a new drug delivery formulation consisting of a novel excipient based on a natural substance isolated from the Aloe Vera plant (not currently approved by the US FDA for use in any drug product for any route of administration) that both retains the drug at the local skin application site and enhances drug penetration into the skin. Local administration of the drug avoids exposing the whole body to the drug; however, being able to retain the drug at the site is problematic since current drug delivery technologies result in short durations of exposure, low local drug exposure, and/or are not user friendly (e.g., leave a greasy film over the application areas, have an undesirable odor, slow down or complicate wound healing etc.). Company ABC's approach uses a novel drug delivery formulation that has major advantages in working with a variety of different drug substances (active ingredients), such as antibiotics and chemotherapeutic drugs.

Company ABC proposes to address a problem with the activity of antibiotics and chemotherapeutic agents used to treat local skin conditions (e.g., infection or cancer). Currently, Company ABC has developed laboratory batches of their drug delivery formulation and prepared formulations containing a variety of antibiotics and chemotherapeutic agents. They have conducted preliminary preclinical studies in mice models to demonstrate that their drug delivery technology provides significantly increased retention of the drugs at the application site and enhances drug penetration into the skin. The focus of Company ABC is to not develop new drug substances (active ingredients) but to take existing drugs and improve their efficacy using their drug delivery technology.

Assessment Overview

This section serves as an executive summary that provides a review of the current state (via a Readiness Score) and high-level key recommendations for four technical categories. Key recommendations listed in **bold** on this Assessment Overview page indicate priority recommendations of greatest urgency. More information for each technical category regarding the current state and detailed recommendations are found on the following pages.

Readiness Score Legend:

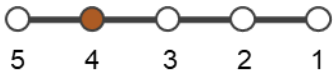
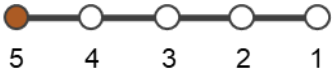
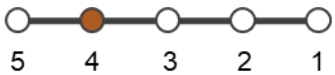
5: ***Not Ready for Commercialization.*** The company has not made significant progress toward commercialization. There is a general awareness of needed steps, but no resources have been identified or committed.

4: ***Planning Readiness.*** The company has begun identifying or obtaining resources to develop commercial readiness and understands what tasks need to be completed to achieve readiness, including early drafts of key documentation.

3: ***Developing Readiness.*** The company has made significant progress on developing readiness for commercialization, including initial drafts of key strategical documentation and an advanced understanding of outstanding tasks and how to obtain remaining resources and external validation to achieve commercialization readiness.

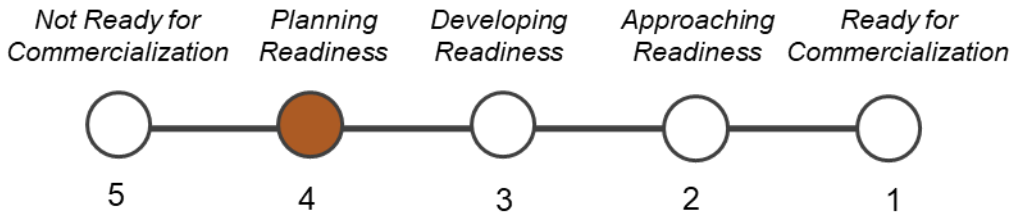
2: ***Approaching Readiness.*** The company only has a few last tasks left to finalize their strategy or required documentation to achieve commercial readiness. Where applicable, external validation of commercialization strategies has been completed.

1: ***Ready for Commercialization.*** The company has completed development of commercialization steps and has validated their strategy with external experts where applicable.

Technical Category & Readiness Score	Key Recommendations
Market Needs/ Competitive Advantage  	• **Identify initial indication for focused development**. • Consider funding strategy based on the initial target indication. • Establish partnerships with drug manufacturers to secure supply
Intellectual Property/ Barriers to Entry  	• **Refrain from future public disclosures until IP is secured.** • **Consult with an IP attorney to protect platform approach.** • Develop individual IP strategies for each product. • Create a structured disclosure approach for future innovations.
Business Model Profitability  	• Address market and competitive landscape activities. • Further explore licensing options.
Manufacturing, Regulatory, and/or Clinical Plan  	• **Hire or contract with a drug development scientist and regulatory expert**. • Develop a scalable production process via fermentation with bioengineered microbes.

Technical Category Details

Market Needs/Competitive Advantages: *Current State*



Strengths	Current Unknowns
• Competitive advantage identified • Identified broad range of target indications	• Unclear market size • Competitors not yet identified • Market point-of-entry is unclear • No partners in place to secure drug compounds

The proposed drug delivery formulation has a proven **competitive advantage** in the marketplace. Currently, technologies that can help patients avoid systemic drug exposure and adverse drug reactions are an unmet need. Company ABC meets market needs through its proposed drug delivery formulation that increases retention of the drugs at the application site and enhances drug penetration into the skin, thus reducing the need for exposure and frequency of adverse drug reactions to current therapies. In tandem with these benefits, **a major advantage** this technology has over its competitors is its **versatility**; the technology works with a variety of different drug substances (active ingredients), such as antibiotics and chemotherapeutic drugs, opening the technology to different drug markets.

Company ABC currently has a **lack of preliminary market data**, which need to be gathered by undertaking activities such as market sizing and indication refinement along with active drug identification whether it is a generic or a branded drug available in the market. The market entry point for this innovation **depends on its initial indication**. The first indication will be dependent on early testing results. If the initial drug API is generic, it will provide ease of entry, but if its branded API, the company will be dependent on a current patent for market entry. Company ABC should also consider that the best market of entry may depend on the chosen point of entry (antibiotics versus cancer), which will further inform strategic planning of the company's first focus product as well as market beachhead.

The current team **lacks knowledge of competitors** in the marketplace. Going forward, Company ABC should consider competing tangential drug delivery modalities such as different ointment, patch, gel, and microneedle injections. Company ABC should extend

this market research into other markets and its competitors, including the veterinary market. Exploring use cases outside of human use and competing drug delivery modalities will guide the company's business strategy; it will be able to determine product and portfolio strategy for drug product development.

Company ABC currently has no partnerships in place to obtain drug compounds for use in the delivery method. The lack of established partnerships means the company **faces a risk of not gaining traction with drug manufacturers** if it is not developing its own drug compounds to be delivered. Establishing partnerships to secure drug compounds early in the R&D process stands to benefit and inform Company ABC when considering pathways to commercialization.

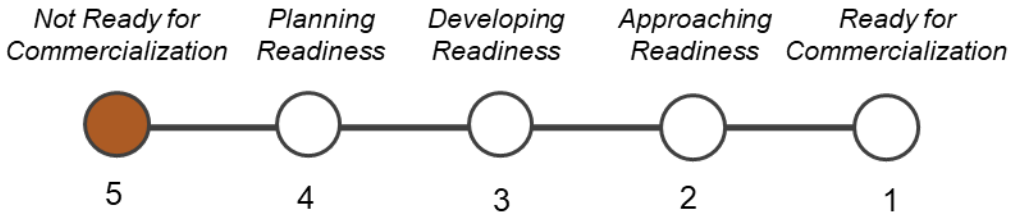
Market Needs/Competitive Advantages: *Recommendations*

Recommendation 1: **Identify initial indication for focused development.**
Based on positive early results of its studies in two indications, the company needs to prioritize a single indication for development. This is not just based on experimental results but will also increase the ability of the team to fundraise (both for non-dilutive fundings and private capital) and other intangible factors.

Recommendation 2: **Consider funding strategy based on the initial target indication.**
Funding requirement of the research and development program is dependent on the identification of an initial target indication. The amount of funds needed for development are based on initial indication, informing the strategy for development of the final drug product. The needed amount can be raised via multiple different pathways, each of which has its benefits and risks. For example, if developing antimicrobial treatment, funding can either be obtained via different federal non-dilutive funding grants (e.g., NIH or BARDA) or through one or more private funders if the expected Return on Investment (ROI) in a given timeframe conforms to the respective investment timeframe preference.

Recommendation 3: **Establish partnerships with drug manufacturers.**
The type of drug compounds that Company ABC can offer in their delivery method will shape its go-to-market strategy. As such, it is in the best interest of the company to establish partnerships with drug manufacturers early on in the development process so R&D can be optimized to specific compounds, as can marketing efforts.

Intellectual Property/Barriers to Entry: *Current State*



Strengths	Current Unknowns
• Multiple potential pathways to IP protection are available	• No IP protection to date • Public disclosure in September 2021 without follow-up • No product name identified and no trademark protection in place

Company ABC would like to pursue IP protection but is unclear about the pathway for IP. It currently does not have an IP counsel or lawyer retained and has only been seeking informal advice through business contacts. As Company ABC has not settled on a product name, there is no trademark protection on the product in place currently.

Company ABC's research team made a public disclosure of the technology at the Global Conference on Pharmaceutics and Drug Delivery Systems in September 2021. During this conference, they received interest from research scientists and representatives of several of the larger drug manufacturing companies. Company ABC expressed concern about IP issues but did not yet pursue additional protection after this event. The company indicated it is planning on resolving IP concerns upon completion of the SBIR Phase I research and development.

Intellectual Property/Barriers to Entry: *Recommendations*

Recommendation 1: **Refrain from future public disclosures until IP is secured.**

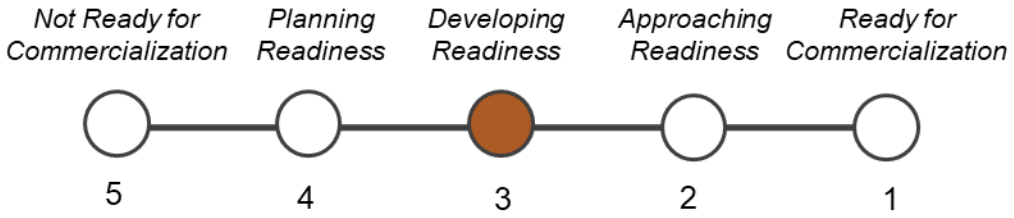
The U.S. Patent and Trademark Office has a grace period of 1 year from public disclosure to file a patent application, which implies that Company ABC should strongly consider filing a patent application before September 2022. Until the patent is filed, Company ABC should limit disclosure of any information related to exact excipient and anything related to method of extraction/formulation to any third party not under NDA.

Recommendation 2: **Consult with an IP attorney to protect platform approach.**
Company ABC would benefit from consulting with an IP attorney who can aid in formulating a patent strategy for the technology. In addition to filing before September 2022 (Recommendation 1), Company ABC should consider a patent strategy for the platform technology and individual treatment strategies, as well as how to secure future formulation patents for different drug products and the process for extracting the natural substance from the aloe vera plant. Furthermore, Company ABC should understand patentability of current and future manufacturing methods. Company ABC has multiple options and potential pathways for IP protection, including the drug-delivery platform itself, the current extraction/purification process, and future bioengineered microbe production process and microbe strain.

Recommendation 3: **Develop individual IP strategies for each product.**
Work with legal counsel and business development individuals for marketing and/or licensing strategy for each product.

Recommendation 4: **Create a structured disclosure approach for future innovations.**
Develop a process for innovation disclosures within the company for future innovations or products as to ensure IP protection can be secured at time of release or shortly thereafter, and only noncritical information is shared during public disclosures. This can limit IP theft or competing companies gaining a competitive advantage.

Business Model Profitability: *Current State*



Strengths	Current Unknowns
• Large and growing market need • Potential for high ROI • Competitive advantage over the market	• Saturated market • Significant investment required to bring to market

Company ABC understands that, in 2020, the global advanced drug delivery market size accounted for $44.02 billion in 2020; and this figure is expected to reach $195.13 billion by 2030–**growing** at a CAGR of 16.0% (source: ABC Market Report). With this market growth and the competitive advantage held by Company ABC, there is a significant opportunity for ROI and reduced need to fundraise if commercialization is executed properly.

However, the technology faces **barriers to entry** that Company ABC should consider, such as uptake due to market saturation with potential alternative formulations for topical application that may already be in the market or in development; the sales force size needed for appropriate ROI; the cost of bringing the technology to market through the development, manufacturing, sales, and approval processes; and which partners it should or can source drug compounds from.

To ensure that Company ABC capitalizes on its competitive advantage and market growth in the face of barriers to entry, the company would benefit from focusing on several key factors: market landscape and competitive landscape activities, licensing strategy, and payment coverage. Addressing the market landscape activities within the advanced drug delivery market will allow Company ABC to prioritize drugs within the market that have indications that their efficacy can be improved–such as a drug that has a high likelihood of adverse drug reactions and is currently difficult to administer topically. Subsequently, exploring the competitive market landscape will help guide the company's business plan and inform pricing strategy. Company ABC should also consider the advantages and disadvantages of licensing the technology as a single product or platform vs stand-alone licensing, as well as whether the technology would be covered by Medicare or Medicaid.

Business Model Profitability: *Recommendations*

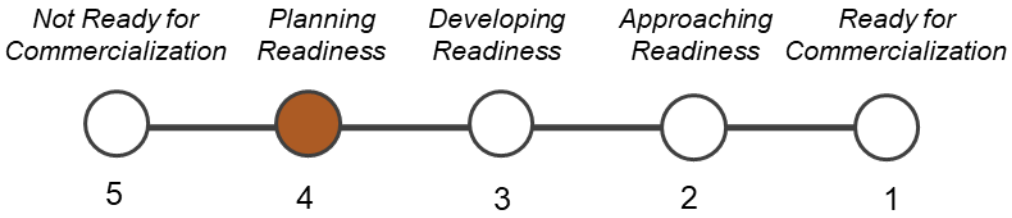
Recommendation 1:

Address market and competitive landscape activities.
To improve profitability of the product and sharpen strategic decisions, Company ABC would benefit from consulting with market experts who can assess market and competitive landscape activities to identify key markets to enter into and competitors within them. Doing so would allow Company ABC to identify drugs within the market that have indications that their efficacy can be improved; identifying key drugs, such as those that have a high likelihood of adverse drug reactions and are currently difficult to administer topically, will aid Company ABC in identifying the market for highest profitability. Consulting with an expert to explore the competitive market landscape will help Company ABC to compete with business plans and price strategies within this identified market.

Recommendation 2:

Further explore licensing options.
To receive the highest return on investment, there are multiple pathways to licensing the technology that can be taken by Company ABC. Each pathway has benefits and risks; it would be beneficial to strategically consider the advantages and disadvantages of each licensing option that is applicable to the technology. These options include licensing the product as a single product versus licensing the product as a platform or licensing the technology as a sole license.

Manufacturing, Regulatory, and/or Clinical Plan: *Current State*



Strengths	Current Unknowns
• Medium to high probability of efficacy and safety • Excipient can be manufactured via fermentation	• Difficult to scale and standardize current manufacturing process • A change in manufacturing processes is likely needed to scale to commercial use

Company ABC's preliminary proof-of-concept formulation work demonstrates a medium to high probability that its technology will improve the efficacy and safety of various drugs in humans. This is supported by preclinical testing in mice models, performed by a CRO, showing enhanced and prolonged drug uptake through the skin after topical application of the drug delivery compound.

Company ABC's current manufacturing approach relies on extraction and purification of the natural excipient from aloe vera plants; however, the team realizes this **approach is costly and complex to scale up** for further clinical studies and commercial production. Furthermore, extraction processes have a **lesser degree of quality control** than other manufacturing processes, which may impact excipient standardization and quality and raise regulatory challenges.

The team has performed an initial literature search to understand options for production of the natural excipient through bioengineered microorganisms (i.e., yeast, bacteria) via fermentation but has yet to select a strain or perform any initial bench scale studies. The team has consulted with a biomanufacturing expert who indicated production of the natural excipient via fermentation is feasible at scale.

The **single biggest regulatory risk** to the company is the lack of FDA approval process for the new excipient. If and when this excipient is approved, Company ABC will need to consider the necessary cost and time to approve. These factors should heavily influence Company ABC in its negotiations with potential partners or its stand-alone strategy for fundraising activities.

Manufacturing, Regulatory, and/or Clinical Plan: *Recommendations*

Recommendation 1:

Hire or contract with a drug development scientist and regulatory expert.
To progress to the next stage of development, Company ABC would benefit from drug development scientist and regulatory consultants/professionals that can help identify gaps/pitfalls and map out a development and regulatory strategy for getting their novel excipient approved by the U.S. FDA using a variety of drug substances (active drug ingredient) for a variety of indications (e.g., skin infection, skin cancer) and using a variety of drugs.

Because Company ABC is focusing on repurposing existing approved drugs, it can pursue the 505(b)(2) NDA pathway.

Recommendation 2:

Develop a scalable production process via fermentation with bioengineered microbes.
Company ABC should begin the initial work (strain selection, bench-scale experiments to optimize process and product titers) to develop a production process for the natural excipient using bioengineered microbes. Development of a bioengineered microbe-based production process not only enables large-scale production and tighter process control but provides another avenue for patent protection and portfolio expansion past the delivery system itself. It also provides a sustainable business model for investors/potential licensees. Once the groundwork is laid by the Company ABC team, scale-up of the process can be performed by a contract manufacturing organization, technology licensee, or other third-party, depending on Company ABC's final business model.