# Pipelines of Promise

| Finalist | Reasons to vote for this company |
|---|---|
| **Arcellx, Inc.** | 1. Imagine a world where patients can get an infusion of CAR-T cells and then go home and safely control their CAR-T cells with weekly SC injections of a protein. That is the new paradigm that Arcellx is developing to revolutionize cell therapy.<br>2. Arcellx is developing first-in-class adaptive immune cell therapies that are readily activated and reprogrammed to provide improved safety, enhanced efficacy, and greater utility across a broader spectrum of oncology and non-oncology indications compared to current CAR-T therapies.<br>3. The Arcellx platform will be amenable to the outpatient setting and accessible to academic AND community physicians transforming the way cell therapy is administered going forward. |
| **Asklepios BioPharmaceutical, Inc. (AskBio)** | 1. Leadership team headed by recognized gene therapy pioneers, Jude Samulski, PhD, and Sheila Mikhail, JD, MBA.<br>2. AskBio is the only fully integrated gene therapy company with next-generation AAV technology, gene control technology, a broad patents and intellectual property portfolio, scaled-up manufacturing and an active portfolio of clinical-stage therapeutic programs.<br>3. Raised $235 million in April 2019 in one of the largest-ever Series A funding rounds in the gene therapy space and has secured milestone and royalty payments from large Pharma deals. |
| **Azitra Inc.** | 1. Azitra is a clinical stage company will begin a Phase II in patients with EGFR-associated rash in 2Q 2020 and a Phase II in patients with atopic dermatitis in 4Q 2020.<br>2. Azitra's science of engineering the microbiome is innovative but supported by substantial data, collaborations with Jackson Laboratory and Yale, and funding from the NIH, NSF, DoD, and DARPA.<br>3. The company is working on both large indications (e.g., eczema) and rare diseases with no or few approved treatment options (e.g., Netherton syndrome). Azitra is addressing these indications with clinical studies planned for 2021. |
| **BioFactura, Inc.** | 1. BioFactura would be of great interest to potential investors and commercialization partners; having an equity position in a biosimilar firm that produces high value biologic drugs at a significant discount to the branded reference drugs.<br>2. Developing a Biosimilar to Synagis (Palivizumab) which is a preventive treatment for RSV infection in premature infants. Over 300K babies die each year as a result of contracting this disease.<br>3. We plan to establish fulfillment centers for the delivery of biosimilar drugs in order to further democratize the patient's ability to procure these therapies, eliminating third party costs, and promoting value-based care options. |
| **Celavie Biosciences** | 1. Celavie's mission is to improve lives and restore hope by advancing novel regenerative therapies, utilizing allogeneic, |

| | |
|---|---|
| Celavie Biosciences<br>STEM CELL TECHNOLOGY TO ENHANCE LIFE | pluripotent stem cells in an undifferentiated and unmodified state, for treatment of Parkinson's disease and other CNS disorders.<br>2. Celavie has promising 5-year exploratory clinical data in 7 Parkinson's disease patients. At five-year evaluation, Unified Parkinson's Disease Rating Scale III scores remained better than baseline in 4/7 patients in OFF condition and in 5/7 patients in ON condition.<br>3. Celavie anticipates beginning Phase I clinical trials for PD and Cerebellar Ataxia SCA1 in the U.S. in 2021. |
| **Cellectar Biosciences, Inc.**<br>CELLECTAR BIOSCIENCES | 1. Our PDC platform is breaking new ground in targeted cancer treatments, potentially providing more efficacious, safe, and patient-friendly treatments to under-served patient populations with rare/orphan designated diseases.<br>2. Our PDC platform can deliver a wide variety of therapeutics to all cancer/cancer stem cells, metastasis, not just those with certain receptors/genes like many current on-market treatments.<br>3. CLR 131, a targeted radioconjugate, demonstrated greater activity in r/r MM than any currently approved therapy for 5th-line patients, while exhibiting very minimal side effects in patients. |
| **Cybrexa Therapeutics**<br>CYBREXA THERAPEUTICS | 1. Dynamic science: Cybrexa is leveraging the low-pH of the tumor microenvironment to selectively deliver anticancer drugs to tumors while sparing healthy tissue.<br>2. Innovation and agility: Cybrexa has taken its lead program from inception to the cusp of filing an IND (in 4Q20) in under 3 years while building out a robust preclinical pipeline, all with a team of less than 20 Connecticut-based scientists and chemists.<br>3. Commitment to community: Cybrexa was formed to realize the potential of a discovery initially made at Yale and has remained committed to the New Haven Community. Cybrexa's two scientific co-founders are Yale Hospital-based physicians who treat both adult and pediatric cancer patients every week. |
| **Cend Therapeutics Inc.**<br>CEND Therapeutics | 1. The company's technology, bifunctional peptide CEND-1 (scientifically aka iRGD), has been studied and validated in more than 200 scientific papers.<br>2. In an ongoing trial in metastatic pancreatic cancer patients, the response rates (PR+CR) are unprecedented (>>2*historical benchmark).<br>3. The technology can be applied to more than 150 solid tumor types, and it has the best potential in hard-to-treat solid tumor types (pancreatic, gastric, liver etc.), and with difficult-to-penetrate therapeutics (antibodies, ADCs etc.) |

| | |
|---|---|
| **Emmaus Life Sciences, Inc.** | 1. The company has dedicated 14 years to develop and approve its sickle cell disease treatment.<br>2. The treatment is first drug in 20 years to address the sickle cell disease indication.<br>3. The company was founded and is still run by the inventor and a leading hematologist who still treats sickle cell patients. |
| **FTG BIO LLC** | 1. mTOR pathway is a clinically validated target for most cancers, rare diseases iand in increasing conditions such as neurodegenerative (Alzheimer, Pick Parkinson's, etc.), metabolic (obesity, type 2 diabetes, etc.) and other diseases. Clinical benefits of mTOR inhibitors i.e. rapalogs (everolimus, temserolimus, sirolimus) in the treatment of cancer & rare diseases i.e. TSC manifestations.<br>2. Selective mTOR inhibitors i.e. TORKinibs provide greater efficacy and safety than PI3K inhibitors by avoiding the PI3K pathway related toxicity. FT-1518 is a promising new mTORC1/2 inhibitor with greater specificity, improved properties, excellent ADME profile and very favorable PK i.e. potential best-in-class selective mTORC1/2 inhibitor, www.ftgbio.com<br>3. Next step, IND submission and starting Phase 1 i.e. partnership, out-license. Strategic development in Oncology & rare diseases, correlation of gene abnormalities with clinical outcome, predictive biomarkers i.e. enrichment population, as single agent and/or combinations i.e. with anit-PD-1 agents. |
| **iCell Gene Therapeutics, LLC** | 1. iCell is developing CAR-T therapies against novel targets with broad IP to address areas with critical needs, including T cell malignancies, Acute Myeloid Leukemia, and Multiple Myeloma.<br>2. iCell is also developing an enhancer technology based on soluble factors that can be used with any CAR cell to improve persistency and efficacy by modulating complimentary host immune system components.<br>3. In addition to promising human data obtained from clinical trials in China, we have initiated clinical trials on several of our products with top cancer institutes in the United States with clinical data expected this year. |
| **Lyndra Inc.** | 1. Lyndra is disrupting how patients take medicine and transforming care. Instead of taking a daily pill, Lyndra's oral, ultra-long-acting, sustained-release platform consistently delivers medicine over the course of a week, month, or longer.<br>2. Lyndra's clinical study results to date support the promise to improve health outcomes, reduce costly complications, increase access globally, reduce complex administration regimens and caregiver burden, and overall reduce total healthcare costs.<br>3. It's hard to think of a disease area where Lyndra's technology wouldn't be a game changer, we're currently prioritizing |

| | |
|---|---|
| | Schizophrenia, Women's Health, Malaria Eradication, HIV, Alzheimer's disease and Opioid Use Disorder. |
| **Magnetic Insight** | 1. Commercialized a breakthrough imaging technology in the life science research market to solve pain points in cell therapy, inflammation and vascular monitoring and diagnostics.<br>2. Full portfolio of nanotechnology solutions for diagnostic imaging and nanoparticle therapy development with localized hyperthermia to deliver targeted payloads or immune modulation.<br>3. Rapid market adoption kickstarted revenue generation within 2 years of inception with 3x growth year over year in research solutions with direct translation to clinical markets. |
| **MetVital, Inc.** | 1. Our drug, "AEO", reduces free glutamate levels in the CNS by up to 40%. Approved for Phase 2 glioblastoma (GBM) clinical work under a commercial IND, and currently in Phase 1 trial for Amyotrophic Lateral Sclerosis (ALS).<br>2. AEO in Phase 2A Clinical has shown an increase in brain glucose uptake in Alzheimer's patients. This may help patients, as glucose uptake decline is directly correlated to cognition decline.<br>3. Oxaloacetate reverses the "Warburg Effect" in GBM, reducing cellular lactate levels by 48%. This may inhibit the growth of many types of cancer. |
| **miRecule, Inc.** | 1. DreamiR Drug Discovery Platform - We have developed a drug discovery platform that drives rationale design of targeted oligonucleotide therapeutics for specific patient groups.<br>2. Cross Functional Team – Our team is composed of serial entrepreneurs with strong expertise in nucleic acid chemistry and drug development. Additionally, our board of clinical, scientific, and business advisers have deep expertise in therapeutic development.<br>3. Operational Advantages – miRecule has partnerships with multiple academic and industry partners, allowing for significant in-kind services, as well as access to both scientific and clinical expertise across multiple programs. This includes a CRADA with the NIH. |
| **Pleiogenix** | 1. Proven Leadership, Development, and Advisory Teams.<br>2. Phase-2 ready orally-available molecule for unmet medical need (NASH/Fibrosis) with huge (> $35 billion) market.<br>3. Flexible business model applicable for drug development for other important indications. |
| **Urigen Pharmaceuticals** | 1. Interstitial Cystitis / Bladder Pain Syndrome (IC/BPS) is a highly unmet medical need with few products in development. Urigen's treatment for painful symptoms will be a major breakthrough for this patient population.<br>2. IC/BPS affects 3 to 6 million patients in the US and predominantly in woman. |

| | |
|---|---|
| | 3. Urigen is looking to also advance a pipeline product for the regeneration of urinary bladder epithelium in patients suffering from radiation and or chemically induced hemorrhagic cystitis. |
| **Versatope Therapeutics**   | 1. Versatope's adaptable vaccine development platform utilizes the power of the recombinant Outer Membrane Vesicles (rOMv's) that could rapidly address emerging viral pandemic threats, such as Covid-19 and pandemic influenza. rOMv's offer antigenic diversity and induce neutralizing antibodies with broad strain recognition, with potential for long-term immunity, using a time release formulation in a single immunization.<br>2. Low cost manufacturing process, using continuously in-line process and de-risked because it is based on currently licensed vaccine products.<br>3. Thermostable formulations will reduce manufacturing costs and enable global distribution without cold-chain supply logistics. |
| **ViCardia Therapeutics, Inc.**   | 1. ViCardia's lead therapy, GP531 is a potent, orally active small molecule that treats mitochondrial dysfunction, the underlying cause of acute and chronic heart failure. GP531 acts as an AMPK agonist improving bioenergetics in the myocardium and improving left ventricular ejection fraction, and promotes biogenesis of mitochondria and revives mitochondria in dormant myocytes.<br>2. ViCardia is led by a highly experienced management team and scientific advisory board with deep expertise in treating heart failure and conducting cardiovascular research.<br>3. ViCardia's protocol for Phase 2 clinical trial is FDA approved, investigator sites are identified, and study kits are being prepared. We are ready to go and expect to meet all endpoints in our protocol. |
| **Zylo Therapeutics LLC**   | 1. Our endocannabinoid-loaded Z-pod solution is showing striking results in a lupus model… Lupus affects women and people of color disproportionately, and has tragic quality-of-life ramifications<br>2. Our nitric-oxide-releasing Z-pod solution is showing compelling results in an Erectile Dysfunction model (where ED is secondary to prostatectomy, a condition that is not treatable with Viagra et al.<br>3. Our technology is disruptive and affordable… and we plan on keeping it that way. |

# Technologies of Tomorrow

| Finalist | Reasons to vote for this company |
|---|---|
| **ARIZ Precision Medicine** | 1. ARIZ Precision Medicine is developing potentially curative therapeutics that selectively deliver fatal genetic messages to cancer cells without damaging healthy cells.<br>2. We target previously 'undruggable' epigenetic changes occurring early in cancerization with siRNA-based therapeutics delivered directly to cancer cells. This combination of drug payload and drug delivery system allows for "Dual Targeting" of cancer, resulting in a safe product and potentially curable outcome for the patient.<br>3. ARIZ recently entered into an equity partnership with co-development partner, Sphaera Pharma, to expand our in vivo studies in animal models of lung and other cancers. We aim to cure cancer. |
| **Autophagy Neurotherapeutic, Inc.**<br>Autophagy Neurotherapeutics, Inc.<br>11350 SW Village Parkway<br>Port St. Lucie, FL 34987 | 1. Autophagy Neurotherapeutics, Inc. is an early-stage biotechnology company located at the Herbert Wertheim College of Medicine at Florida International University (FIU) in Miami, FL. , and is focused on lead optimization and pre-clinical development of small molecule drugs for treating ALS (Amyotrophic Lateral Sclerosis), Alzheimer's disease and other neurodegenerative diseases.<br>2. The technological approach is to modulate autophagy, which is the brain's system for eliminating toxic proteins. It is emerging as one of the best ways to develop drugs for all neurodegenerative diseases.<br>3. Our company is developing 2 drugs, TPI-132 and THPI-244, which look very promising. |
| **BioSensics LLC** | 1. BioSensics has created new paradigms in using wearable sensors in healthcare and revolutionized the medical alert industry by developing technologies that are now being used by thousands of older adults.<br>2. BioSensics solutions encompass comprehensive and validated technologies for collection of digital biomarkers and outcome assessment in healthcare. Our solutions are now empowering novel healthcare initiatives and important clinical trials in a variety of therapeutic areas.<br>3. The BioDigit Database offers the most comprehensive database of disease-specific digital biomarkers. This database effectively eliminates the need for early studies and phase 0 in many therapeutic areas. |
| **Boston Immune Technologies and Therapeutics, Inc.** | 1. Novel approach to TNF Superfamily antagonism.<br>2. Solid preclinical data improving check point inhibitor response.<br>3. Lead TNFR2 mAb near to clinic. |
| **Boundless Bio** | 1. Boundless is seeking to treat one of the greatest unmet needs in cancer, gene-amplification driven tumors, which account for one |

| | |
|---|---|
| | quarter of cancer cases, have a dismal prognosis, and are resistant to all standards of care<br>2. Boundless has unlocked the driver biology, on extrachromosomal DNA (ecDNA), that enables gene amplifications to arise, thrive, resist treatment, and pass from cell to cell<br>3. From this ecDNA insight and focus, Boundless has identified multiple, unprecedented cancer targets that it is actively drugging and enables us to pursue previously "undruggable" cancers, like C-Myc amplified and wild-type KRAS amplified tumors |
| **Cardea Bio** | 1. Cardea's CRISPR-Chip™ technology became the June 2019 cover-story and most-read paper in Nature Biomedical Engineering, with the headline: Amplification-free nucleic-acid testing.<br>2. The Cardean Biology-gated Transistor™ tech-infrastructure enables near-instant, multi-analyte measurements of DNA, RNA and proteins from a single sample via a handheld electronic device.<br>3. This optical-free infrastructure combines whole biology with biocompatible electronics, allowing Cardea's partners to develop new generations of (bio)technology products with faster and deeper insight into systems biology. |
| **Clara Diagnostics, Inc** | 1. Huge Impact! We have the platform enabling a new generation of biotechnology solutions around naturally derived exosomes with applications in cancer immunotherapy, neurodegenerative diseases, targeted drug delivery, and more with our proprietary immune-isolation technology moving research to patient.<br>2. Purity and Quality! We're solving an industry-wide problem by providing the only isolation platform able to isolate pure exosomes from any source and we do it at scale.<br>3. Traction! Already working with many large biopharma companies interested in getting exosome solutions to market. |
| **Efferent Labs, Inc.** | 1. Efferent Labs has invested years of research and development and company is poised to take product to first market in 2021. This product disrupts the pre-clinical market by allowing real time data of in vivo information.<br>2. Our products have multiple verticals starting in pre-clinical drug development that will provide a path to reduce drug development costs. Later verticals include disease specific uses for human drug dosing (chemotherapy, immunotherapy) as well as other uses.<br>3. Novel in vivo cellular monitoring device that can, as a system, provide proactive dose response information to physicians. |
| **Lindy Biosciences** | 1. Lindy Bio is developing a formulation technology to enable fast, simple, at-home drug administration for patients suffering from debilitating chronic diseases. Our dehydration technology enables highly concentrated protein suspensions, allowing higher dosing in smaller volumes, ultimately reducing the time patients spend taking medication.<br>2. Patient-Centric & Patient-First: We return control and quality of life to patients (convenience, compliance, comfort) with simpler drug delivery – converting IV injectables (hours in clinic) to |

| | |
|---|---|
| | SC injections (seconds at home).<br>3. This benefits Pharma, allowing high-dose molecules to reach the market and extending patent life, and benefits Payers with reduced healthcare costs. |
| **Meru Biotechnologies** | 1. Meru Biotechnologies is offering a unique mix and read ligand binding assay that is quantitative and label-free.<br>2. Meru's assay platform is not compromised by matrix effects, meaning that the assay in quantitative in complex biological samples.<br>3. Meru's technology can detect and quantitate the following analytes and drug modalities: proteins & metabolites, small molecules, biologics, RNA, DNA, and ions. |
| **MyoGene Bio** | 1. Dr. Courtney Young, CEO of MyoGene Bio, is working to save the life of her cousin Christopher and thousands of other boys afflicted with Duchenne muscular dystrophy.<br>2. MyoGene Bio's gene editing therapy aims to treat ~50% of Duchenne patients to stabilize the disease progression and improve life for the patient, family and community.<br>3. MyoGene Bio's innovative gene editing therapy uses cutting edge technology to produce the best possible treatment for these boys. |
| **Nanochon** | 1. We are young, hungry and motivated!<br>2. Nanochon is on the cutting edge of 3D printing and tissue engineering.<br>3. Nanochon would be a huge clinical improvement and massive cost reduced in the ortho space. |
| **Nanopec Inc.** | 1. Early cancer detection is the key to successful patient outcomes. NANOPEC's technology allows early cancer diagnostics by detecting lower cancer biomarker concentrations than incumbent in vitro diagnostics.<br>2. The high cost of in vitro diagnostics limit their use in patient health management. NANOPEC products lower their costs by reducing the amount of expensive reagents such as detection antibodies, by up to 80%.<br>3. False negatives diagnostics cause patients not to get the right treatment when needed the most. NANOPEC's disruptive technology platform enhances fluorescence signal to noise ratios by up to 2000%, eliminating false results. |
| **Neurgain Technologies** | 1. Our Company is seeking to treat via gene therapy a major medical and social problem - Neuropathic pain.<br>2. Our delivery technology enables companies that are seeking to treat spinal cord injury and motor discovers such as ALS with either gene or cell therapy. |

| | |
|---|---|
| | 3. Our technologies create curative therapies to what has been either incurable disease or those diseases that require chronic therapies with poor outcomes. |
| **Orbital Transports** | 1. Microgravity has proven drug development benefits.<br>2. Orbital Transports provides low-cost satellites for in-space microgravity research platforms (which can be used with organ chips/lab-on-a-chips), and handles the entire mission so you can focus on your experiments.<br>3. If necessary, your research samples can be returned from space to your laboratory. |
| **Palisades Therapeutics/Pop Test Oncology LLC** | 1. Therapeutics for unmet needs. Palisades Therapeutics has a platform of compounds with a new mechanism of action that are demonstrating in vitro and in vivo efficacy across a spectrum of difficult to treat disease states including oncology, virology, addiction.<br>2. Clinical stage and early stage platforms. Our platform consists of a small molecule that has completed Ph2 studies and several pre-clinical compounds.<br>3. 100% of funds raised go to the projects' development. We have assembled a highly experienced team across multiple functional areas; all are working without salary to prove out programs and drive towards partnering / commercialization. |
| **ReForm Biologics, LLC** | 1. ReForm Biologics has developed an innovative approach to biotherapeutic formulation that can generate market differentiating formulation that enable superior dosing and delivery for the benefit of patients<br>2. ReForm has signed a strategic partnership with MilliporeSigma, a life science business of Merck KGaA, Germany for the development and commercialization of our technology.<br>3. ReForm is developing a proprietary pipeline of patented formulations and has 5 US issued patents that protect our internal programs and our partners who license our formulation technology for their biologics. |
| **unlearn.ai** | 1. Unlearn.AI has developed the first machine learning (ML) platform for creating Intelligent Control Arms with Digital Twins through its proprietary DiGenesis™ process.<br>2. Intelligent Control arms allow drug developers to dramatically reduce therapy development time, while lowering the risk of trial failure, thereby increasing confidence in clinical trial results.<br>3. Unlearn is working closely with biopharmaceutical and medical device companies as well as regulators to ensure its methods meet the highest scientific and regulatory standards. |
| **Varigen Biosciences Corporation** | 1. Varigen is leading the renaissance in natural product drug discovery and optimization. Rather than screening the molecular output of *cultured* microbes (been there, done that), Varigen isolates the gene sequences that direct the cellular machinery to manufacture biologically-relevant secondary metabolites. |

| | |
|---|---|
| Varigen BIOSCIENCES The BIG DNA Company | 2. Varigen's suite of proprietary molecular genetics tools and deep in-house expertise in the manipulation and expression of large biosynthetic gene clusters (BGCs) grants us exclusive access to the enormous repository of genomically-encoded molecules overlooked in previous fermentation-based screening programs.<br>3. Having identified thousands of BGCs in our libraries, we are screening them for disease-related bioactivity. |
| **Vincere Biosciences, Inc**<br>VINCERE | 1. First in class potential for small molecules that enhance mitophagy for Parkinson's and many other potential indications.<br>2. Patented computer simulation of patient cells predicts efficacy for complex disease.<br>3. Unparalleled in-house team blending science and technology to rapidly discover and develop new drugs. |