

ETX Pharma, Inc.

***Clinical Stage Biotech Company
Developing Drugs for
Large Unmet Gastrointestinal (GI) Markets***



Dr. Craig M Liddell – President & CEO

www.etxpharma.com

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Why ETX Pharma?



- **Later Clinical Products Lower Risk & Provide a Path to Rapid Exits**
- **Disease modifying drugs – first in class – safe for chronic use**
- **Addressing large markets with unmet medical needs**
 - *Phase 2/3 ready drug for gastroparesis*
 - *Accelerated 505 (b)(2) approval path & proven efficacy & safety*
 - *Go to market in 2021 and ramps to \$1B+ annual revenue in 5 years*
 - *Phase 2 ready drug for Crohn's Disease*
 - *Oral drug to compete with Humira® (\$9.3B, 2012)*
 - *Out-license in 4 years (2020)*



Pipeline

➤ **Mix of Repurposed Drug, Clinical NCE & Preclinical NCE**

Program	Development Stage		Regulatory Pathway	Indication	Top Line Data
	Pending IND	Phase 1	Phase 2		
ETX-101			505(b)(2) (Accelerated Registration)	Diabetic/Idiopathic ¹ Gastroparesis	2020 Phase 3
ETX-101			505(b)(2) (Accelerated Registration)	Pediatric/Orphan (Type 1 Diabetes) Gastroparesis	2022 Phase 3
ETX-201*			NCE	IBD – Crohn’s Disease	2020 Phase 2
ETX-301		Pre-IND	NCE	Diabetic/Idiopathic ¹ Gastroparesis	2020 Phase 1

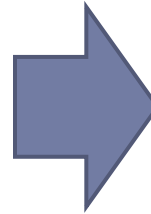
*ETX Pharma rights to ETX-201 are pending negotiation



ETX-101: Repurposed for Gastroparesis

Kuvan®

- approved in 2007 for PKU¹
- pediatric approval
- up to 9 years controlled clinical studies
- *13,000 patients in US use drug on a chronic basis*



ETX-101

- worldwide exclusive GI license
- Treats nitric oxide deficiency
- phase 2b/3 ready
- global commercial deal
- cGMP Drug in 120 days
- Up to 7 yrs exclusivity – Pediatric & Orphan
- *~3,000,000 patients in U.S.*

PKU¹ Indication

\$150M/year



Worldwide Exclusive GI Method of Use License

Worldwide Exclusive novel manufacturing patents

Worldwide Exclusive patents to tablet formulation



**Market Launch
Gastroparesis
in
2021**

~\$1+B/year

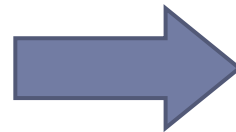
¹ Phenylketonuria (BH4-Dependent)



ETX-201*: Oral TNF Production Inhibitor

➤ Humira®

- approved in 2002
- over \$9B in 2012
- biologic
- *intravenous Drug*
- *life threatening side effects*



➤ ETX-201

- worldwide Acquisition
- 2030 COM patent expiration
- over 300 patients prior I.V.
- ORAL – small molecule drug
- non-biologic, safe
- phase 2 ready

Phase 1 Completed



Worldwide Exclusive
Method of Use
Patent(s)

Worldwide Exclusive
composition-of-
matter patents

Worldwide Exclusive
agreement to acquire

**Outlicense at
end of phase 2
(2020)
(phase 3 ready)**



ETX-301: Completely Novel Therapy

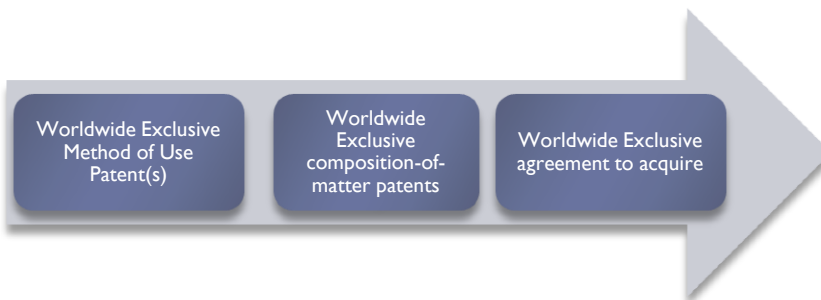
➤ **Gastroparesis**

- recognized unmet market
- poor therapeutic options
- recent medical evidence
 - Nitric oxide based MOA
 - clinical evidence growing



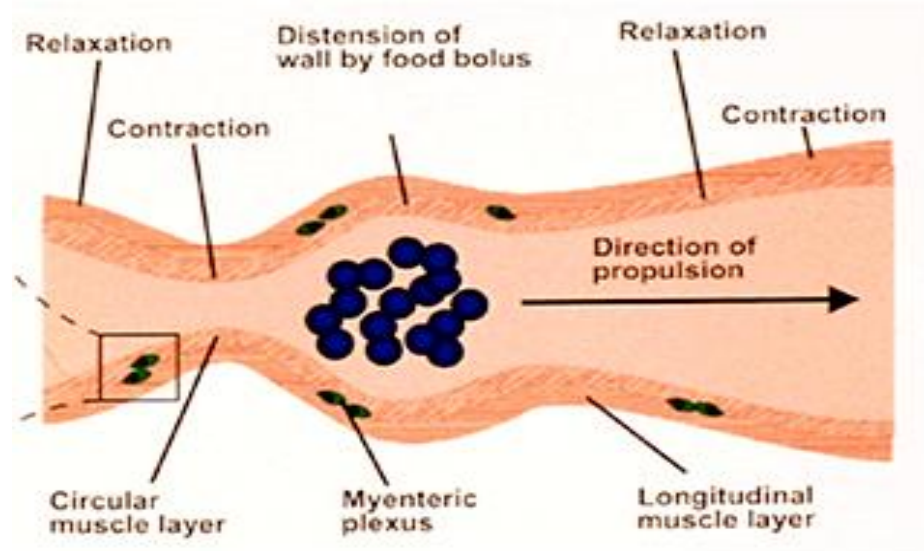
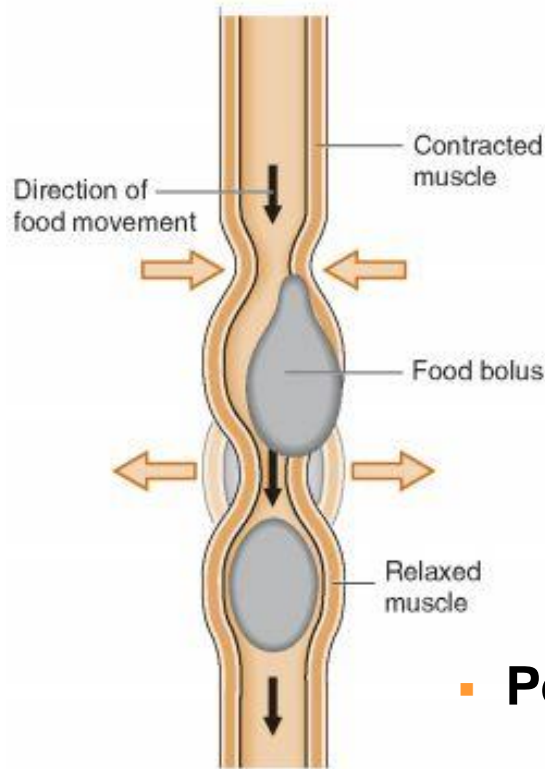
➤ **ETX-301**

- worldwide Acquisition
- 2027-2035 patent expiration
- ORAL – nitric oxide donor
 - non-absorbed polymer
 - novel MOA





Active relaxation is critical for peristalsis – mediated by nitric oxide

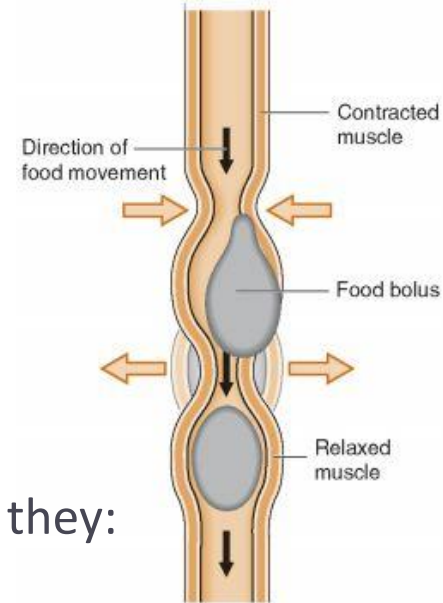


- **Peristalsis requires the coordination of BOTH:**
 - Active Contract Signal
 - Active Relax Signal



ETX-101/301 Differentiation

- ▶ **ETX-101 & ETX-301 –**
 - ▶ Nitric oxide is the **KEY** molecule triggering muscle relaxation
- ▶ **Gastroparesis is CAUSED by loss of nitric oxide “relax” signal**
 - ▶ Disease is NOT caused by loss of the “contract” message
 - ▶ Disease is caused by the loss of the “relax” message
- ▶ To date most gastroparesis drug candidates failed because they:
 - ▶ 1) target only the “contract” message
 - ▶ 2) target high-level hormones and peptides that incorrectly assume the nitric oxide “relax” message is functioning properly¹
- ▶ **ETX-101 & ETX-301 are First-in-Class drugs in development that directly address the nitric oxide loss in mild to end-stage gastroparesis**





ETX-101/301 Positioning

▶ **Gastroparesis (Gastric Stasis)**

- ▶ **Develops over decades as the nitric oxide message is progressively lost**
 - ▶ **Develops into severe end stage disease when nitric oxide is no longer produced**
-
- ▶ **ETX-101**
 - ▶ Restores partially to severely degraded nitric oxide message to restore normal function

 - ▶ **ETX-301**
 - ▶ Replaces the nitric oxide message in patients with end-stage disease and who have completely lost the nitric oxide messaging system



IBD Market Opportunity

▶ **Inflammatory Bowel Disease – IBD**

- ▶ Ulcerative Colitis (IBD-UC)
- ▶ Crohn's Disease (IBD-CD)
 - ▶ ~4 MM persons worldwide have UC or CD
 - ▶ ~1.4MM of these cases occur in the United States.

▶ **IBD (both CD and UC) is frequently misdiagnosed**

- ▶ IBD in the US accounts for more than:
 - 700,000 physician visits
 - 100,000 hospitalizations, and
 - major disability in 119,000 patients

▶ **Over the long term, up to 75% of patients with CD and 25% of those with UC will require surgery.**



IBD-CD Market Opportunity

- ▶ **Three drug classes are used to treat CD:**
 - ▶ Front line and Second Line therapy in mild-moderate CD: Corticosteroids
 - ▶ Third Line (Next Generation): Biological TNF¹ Inhibitors (Remicade, Humira, and Cimzia) and two anti-integrin antibodies (Tysabri and Entyvio)
 - Adjunct second and third line: Immune modifiers (Azathioprine, 6-MP, and Methotrexate)
- ▶ **ETX-201 is an *oral TNF¹ synthesis inhibitor* for 2nd or 3rd line**
- ▶ **ETX-201 is a potential disease modifying therapy**
- ▶ **Inhibits multiple components of the inflammatory processes leading to IBD in addition to TNF: iNOS, IL1, IL6 and P38.**
- ▶ **Competitive drugs in development are either intravenous or SQ and therefore difficult for patient administration or do not treat the breadth of inflammatory targets of ETX-201**
- ▶ **Many competitive drugs are biological and therefore immunogenic; ETX-201 is highly unlikely to be immunogenic**

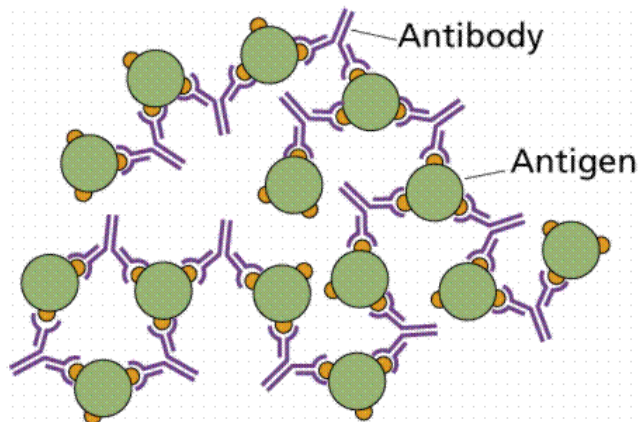
¹TNF – Tumor Necrosis Factor



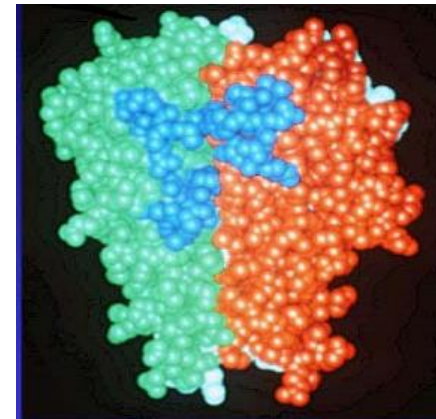
ETX-201 Differentiation

▶ ETX-201

- ▶ Intravenous anti-TNF drugs such as Humira® and Remicade® are antibodies that antagonize TNF throughout the body leading to severe side effects, such as cancer & susceptibility to infection
- ▶ ETX-201 acts locally in the GI tract and inhibits TNF synthesis (without completely shutting it down) for effective disease control without systemic side effects



Anti-TNF ANTIBODIES strongly bind the antigen (TNF) completely shutting it down



ETX-201 is a small molecule Inhibitor of TNF synthesis reducing its activity¹



ETX-201 Positioning

- ▶ **Inflammatory Bowel Disease (IBD) - CD & UC**
 - ▶ Develops as result of over-sensitivity to normal gut microbiome
 - ▶ Develops into severe end stage disease when inflammatory processes are uncontrolled
- ▶ **ETX-201**
 - ▶ Is an oral anti-inflammatory drug that inhibits but does not completely shut down inflammatory processes required to protect the body from bad microbes and cancerous cells



Intellectual Property/Exclusivity

➤ **ETX-101**

- U.S. Method of Use patent to 2030
- Worldwide supply manufacturing patents to 2031
- Worldwide formulation patents to 2031
- Statutory Exclusivity - New Indication, Orphan and Pediatric up to 7 years

➤ **ETX-201**

- Composition of Matter with protection to 2030

➤ **ETX-301**

- Composition of Matter Patents
 - US 8,894,985 and US 7,829,553 with protection to 2027
- Pending Provisional patents





: Drug Product Protection

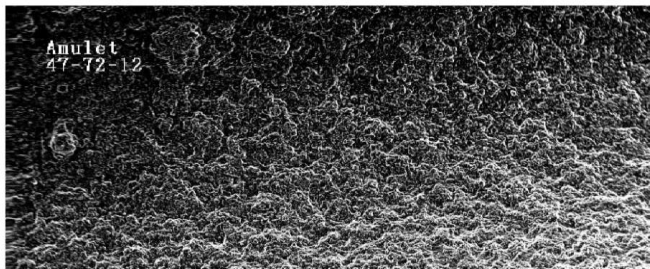
- **ETX-101 Protection to 2031**
 - **1) Patent Protection to 2031**
 - **ETX has an exclusive license to U.S. method-of-use patent to be listed in Orange Book**
 - **2) Exclusive license to worldwide manufacturing patents that expire in 2031**
 - **3) Exclusive license to worldwide formulation patents that expire in 2031**
- **ETX-201 Protection to 2030**
 - **1) Composition of Matter Patent Protection to 2030**
- **ETX-301 Protection to 2036**
 - **1) Composition of Matter Patent Protection of enabling patents to 2025**
 - **2) Composition of Matter Patent Protection of product patents to 2036**



Near Term Out-Licensing Opportunities: Antimicrobial Hemostatic Wound Dressings

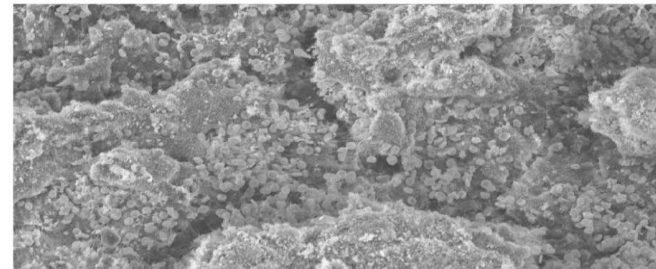
- **ETX-302 for wound dressings**
 - High flux Nitric Oxide is antimicrobial
 - Hemostatic and wound healing properties
- **Novel Formulations and IP**
 - Biostable or biodegradable powder
 - Powder contained in a textile/porous bag
 - Biostable or Biodegradable Spray or Polymer Film Dressings
- **Product Concepts**
 - Skin dressing – Military & Diabetic Ulcer
 - Applied to the organs after trauma or surgery e.g. liver, lungs, and spleen.

Figure 1 Surface of hemostatic film 70 X



Simple film-based formulation

Figure 2 10 minute exposure of hemostatic film in canine AV Shunt 350X



Immediate and Significant Thrombus





Leadership Team

➤ **Craig M. Liddell PhD** – President & CEO

- Paradigm Genetics/Icoria, Artestian and Amulet - \$45M IPO; \$29M equity raised; \$28M+ grants and contracts.
- Three successful drug programs in development.

➤ **Christine D. Copple PhD** – EVP, COO

- Therabron Therapeutics, Neuronascent, ASMR V Fund, Metabiomics, Ammonett,
- Founder Neuralstem (market cap \$340M), Founder Microfluidics, ASM Venture Fund
- CytImmune Sciences, Creatv MicroTech, 60 Degree Pharma, Origent Data Sciences

➤ **Peter Gordon** – Interim CFO - President & CEO of 1st US Capital Inc

- Recent successful exit of Melanovus Oncology, Inc to a public Company in December 2014.
- Formerly Chief Financial Officer, Corporate Secretary, Co-Founder and a Director of a public reporting therapeutic and diagnostic cancer firm and raised over \$61 million in seven private rounds through accredited investors.
- Over \$1B raised in multiple Companies.

➤ **Pankaj Jay Pasricha MD** – Chair, CSAB

- Professor of Medicine, Johns Hopkins Medicine
- Director of Johns Hopkins Center for Neurogastroenterology

➤ **Michael Helmus PhD** – EVP, MedTech Strategy and Innovation

- 35 years developing/commercializing Medtech and combination drug delivery systems at Pfizer, Baxter, Boston Scientific, Advance Nanotech, Amulet Pharma (Vascular grafts, heart valves, heparin coated implants, tissue sealants, drug eluting devices and stents)
- Due diligence, developing commercialization strategies of potentially disruptive technology, White Space Innovation, 44 US Patents
- Extensive experience in all phases of managing medical device development projects from inception to market; Member SABs

➤ **Ralph T. Scannell PhD** – VP, Chemistry

- 28 years of drug discovery and development experience in the pharmaceutical/biotechnology industry including A.H. Robins Pharmaceuticals; Ethyl Corporation; CytoMed, Inc.; UCB Pharmaceuticals; Amulet.
- Five successful drug programs: three in development and three IND's



Clinical/Scientific Advisors



➤ **Pankaj Jay Pasricha MD – Chair, CSAB**

- Professor of Medicine, Johns Hopkins Medicine
- Director of Center for Motility Disorders & Digestive Diseases
- Director, Johns Hopkins Center for Neurogastroenterology



➤ **Linda Nguyen MD**

- Clinical Associate Professor, Medicine - Gastroenterology & Hepatology
- Director, GI Motility & Neurogastroenterology, Stanford U Medicine

➤ **Robert Rauli PhD**

- Founder, Amulet Pharma Inc

➤ **William Sandborn MD**

- Professor of Medicine, UC San Diego
- Chief, Div of Gastroenterology, U of CA San Diego, School of Medicine

➤ **Bruce E. Sands MD MS**

- Dr. Burrill B. Crohn Professor of Medicine, Mount Sinai Health System and Icahn School of Medicine at Mount Sinai, New York, NY
- Fellow - American Gastroenterological Association
- Fellow - American College of Gastroenterology

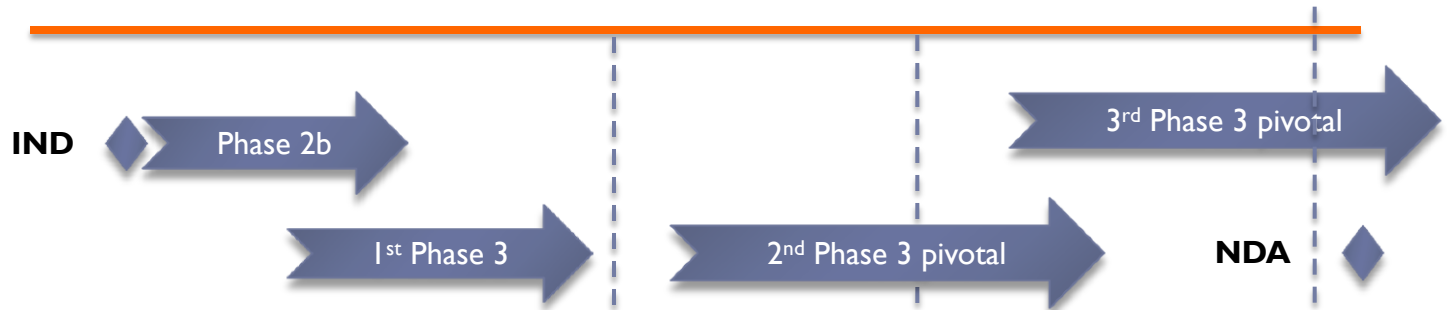




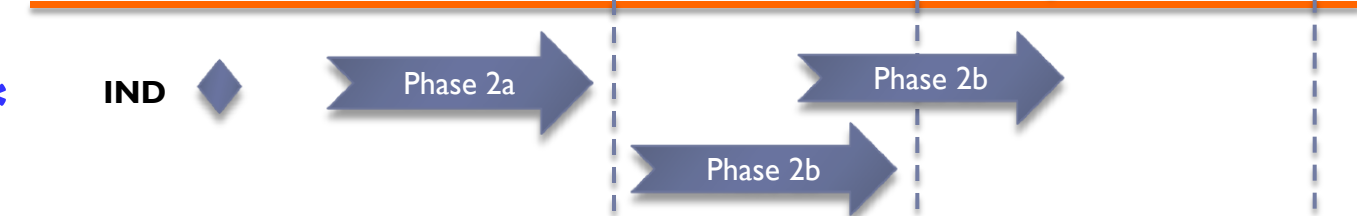
Timeline 2016-2021

2016 2017 2018 2019 2020 2021

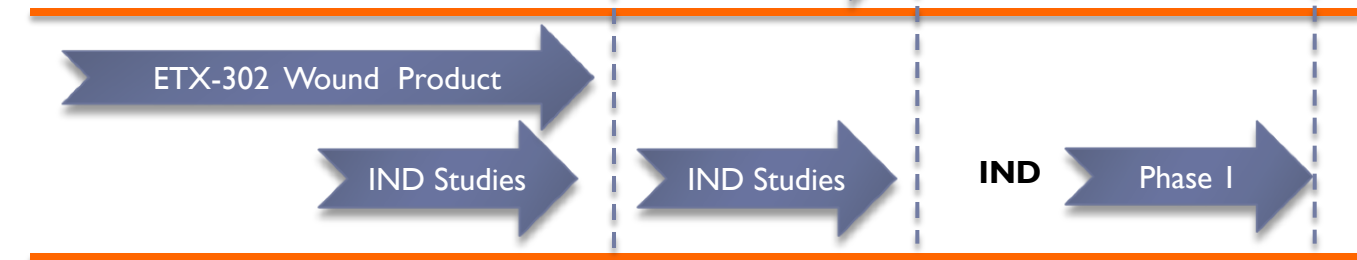
ETX-101



ETX-201*



ETX-301



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