

# Reimagining Molecules to Advance Medicine

Corporate Presentation – November 2024



#### Safe Harbor



#### SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This document contains forward-looking statements concerning Eton Pharmaceuticals, Inc. ("Eton", the "Company," "we," "us," and "our"). The words "believe," "may," "will," "potentially," "estimate," "continue," "anticipate," "intend," "could," "would," "project," "plan," "expect" and similar expressions that convey uncertainty of future events or outcomes are intended to identify forward-looking statements. These forward-looking statements include, but are not limited to, statements concerning the following:

our future financial and operating results;

• our intentions, expectations and beliefs regarding anticipated growth, market penetration and trends in our business;

• the timing and success of our plan of commercialization;

our ability to successfully develop and clinically test our product candidates; and
our ability to file for FDA approval of our product candidates through the 505(b)(2) regulatory pathway.

These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in "Risk Factors" section of the Registration Statement on Form S-1 filed Eton with the Securities and Exchange Commission on September 25, 2018. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this document may not occur and actual results could differ materially and adversely from those anticipated or implied in our forward-looking statements. You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in our forward-looking statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances described in the forward-looking statements will be achieved or occur.

This document contains only basic information concerning Eton. Because it is a summary it does not contain all of the information you should consider before investing.

Before you invest, you should read the prospectus in that registration statement and other documents Eton has filed with the SEC for more complete information about Eton and this offering. You may get these documents for free by visiting EDGAR on the SEC Web site at www.sec.gov. Alternatively, Eton, any underwriter or any dealer participating in the offering will arrange to send you the prospectus if you request it by calling National Securities Corporation toll-free at 1-800-832-6084.

Disclaimer: Products discussed in this presentation are under development and not yet approved by the FDA. The information presented here is to the best of the company's current knowledge and assumptions, which may be different from the labeling and conditions provided by the FDA upon approval, which approval itself is not guaranteed. In no event should this information be construed as marketing or promotion, or providing directions for use of any product, all of which are contingent on FDA approval.

# **Investment Highlights**



Dedicated to developing and commercializing pharmaceutical products to address unmet needs in patients suffering from extremely rare conditions

**ULTRA-RARE DISEASE FOCUS** 



Focus on bringing treatments to patients with the rarest of conditions (<10,000 patients)

**6 COMMERCIAL PRODUCTS\*** 



Therapeutic areas of pediatric endocrinology and metabolic genetics

**3 LATE-STAGE PIPELINE CANDIDATES** 



**3** additional products in late-stage development, including 1 with a **Q1 2025 PDUFA** date

**RAPIDLY GROWING REVENUE** 



**15** straight quarters of sequential revenue growth since launch of ALKINDI SPRINKLE

STRONG FINANCIAL POSITION



Positive net income in Q3 2024 and \$20.3 million of cash on hand to support new product acquisitions and R&D\*\*

<sup>\*</sup>Includes pending acquisition Increlex®, expected to close near year-end 2024
\*\* As of September 30, 2024

# Why Ultra-Rare?





#### **LARGE OPPORTUNITY**

More than 5,000 rare diseases have no FDA-approved treatment option



#### **MEANINGFUL IMPACT**

Eton prefers markets where it can provide the first or only FDA-approved treatment option for patients



# TARGETED COMMERCIALIZATION

Small patient populations have small prescriber bases, enabling efficient commercialization of multiple products simultaneously



#### **LIMITED COMPETITION**

Many companies are unwilling to dedicate development and commercial resources to products that treat small patient populations

#### **Eton's Ultra-Rare Disease Products**

Carglumic Acid tablets for oral suspension 200 mg

~100

increlex\*
(mecasermin) injection 10 mg
(pending\*)

Nitisinone Capsules

Betaine Anhydrous

ET-600

**EGOLIKE** 

Alkindi Sprinkle

& ET-400

Traditional
Drug
Products

<200,000

**FDA** 

**Orphan** 

**Definition** 

Products
200,000+

Est. US Patient Populations

(pci

~200

\*) C

200-300

1,000-2,000

<5,000

~8,000

~10,000

#### **Ultra-Rare Commercialization**



#### Eton's commercial strategy is designed to serve the unique needs of ultra-rare patient populations



**Concentrated prescriber bases.** Small number of specialists within pediatric endocrinology and metabolic genetics allows for an efficient twelve-person sales force to cover targets across the country.



**Meaningful partnerships with patient advocacy groups.** Eton works closely with patient and caregiver groups to advance awareness, education, and patient support resources.



**Active advisory board of key opinion leaders.** Regular engagement with physicians and healthcare professionals leading the research and treatment of ultra-rare conditions.

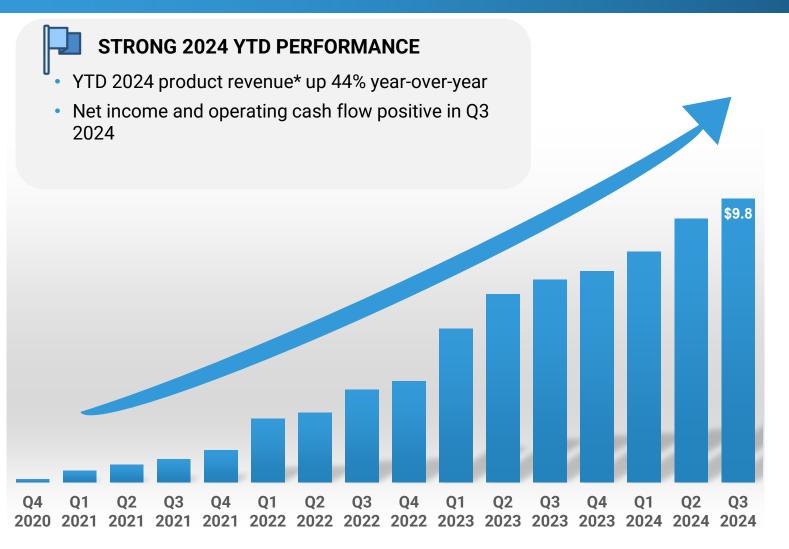


**Eton Cares patient support program in place for** <u>all </u>**products.** \$0 co-pay for commercial patients, quick-start program, nurse hotline, monthly patient check-in, and exclusive distribution through high-touch specialty pharmacy.



# 15 Straight Quarters of Product Revenue Growth





# 3 PART STRATEGY FOR LONG-TERM REVENUE GROWTH

Grow current portfolio

Continued organic growth of ALKINDI SPRINKLE (2034 IP), Carglumic Acid, Betaine, Nitisinone, and PKU GOLIKE

New product launches

Anticipated future launches of ET-400 (2025) and ET-600 (2026)

Product acquisitions & licensing

Increlex acquisition expected to add revenue in Q1 2025. Actively engaged in discussions to acquire or license additional products

Chart: Quarterly product sales and royalty revenue in millions

\*Product sales and royalty revenue, excludes licensing revenue

# Path to 10 Commercial Products by 2025



CMC Development

Phase III/ Pivotal Study NDA Submission

Commercial

- 1 ALKINDI SPRINKLE® Pediatric Adrenal Insufficiency
- 2 Increlex® Primary Insulin-Like Growth Factor 1 Deficiency\*
- 3 Carglumic Acid Hyperammonemia
- 4 PKU GOLIKE® Phenylketonuria (PKU)
- 5 Nitisinone Hereditary Tyrosinemia Type 1 (HT-1)
- 6 Betaine Anhydrous Homocystinuria
- 7 ET-400 Pediatric

2/28/2025 PDUFA date

8 ET-600 – Diabetes Insipidus

Q1 2025 NDA submission\*\*

9 ZENEO® - Adrenal Crisis

Long-term goal of 10 commercial rare disease products by end of 2025

8

Potential products from existing portfolio & pipeline by 2025

2+

Products from future acquisitions to reach 2025 goal

#### **ALKINDI SPRINKLE®**



# ALKINDI SPRINKLE® is a proprietary formulation of hydrocortisone sprinkles that is FDA approved as a replacement therapy for adrenocortical insufficiency (AI) in patients under 17 years of age

- First and only hydrocortisone treatment specifically designed to help provide accurate dosing for newborns and children with adrenal insufficiency. Addresses unmet need for accurate, low-dose treatment.
- Eliminates the need for patients to split tablets, crush tablets, or utilize compounded suspensions.
- Three orange-book listed patents extending to 2034.

# Pill Splitting Pill Crushing RISK OF OVERDOSE OR UNDERDOSE\*



#### NDA Submitted: ET-400



#### NDA accepted for ET-400 (hydrocortisone oral solution) with February 28, 2025 PDUFA date



**Highly requested dosage form.** ET-400 will provide an additional oral hydrocortisone treatment option. Patients and caregivers have expressed strong demand for an FDA-approved liquid formulation of hydrocortisone, particularly among infants and young children.



**Gives Eton ability to capture a greater percentage of the oral hydrocortisone market.** Sales of ET-400 expected to drive significant revenue growth.



**Proprietary formulation.** Eton developed a patented proprietary formulation to create a room temperature stable oral solution. Patent coverage through 2043.



**NDA accepted by the FDA.** PDUFA date set for February 28, 2025. Eton anticipates initiating production of commercial product in Q4 2024, allowing for a commercial launch promptly after the expected approval.

# Pending Acquisition: INCRELEX®



Eton executed an agreement to acquire Increlex® global rights from Ipsen in October 2024.

Transaction expected to close near year-end 2024



**Treats Severe Primary IGF-1 Deficiency, which causes short stature in children and adolescents.** It is estimated that approximately 200 patients in the United States and 900-1,000 patients in Europe live with SPIGFD.



**Compelling strategic fit.** Strong overlap with Eton's existing Pediatric Endocrinology presence. >80% of Increlex prescribers are existing Eton targets for Alkindi Sprinkle.



**Attractive long-term growth potential.** Eton believes SPIGFD is under-diagnosed and under-treated in the United States. Product's biologic manufacturing process protects long-term revenue.



**Immediate commercial impact.** Post closing, Eton will immediately commercialize the product in the U.S., Ipsen will continue to distribute in Europe for 6-month transition period. Ipsen reported 2023 revenue for Increlex of approximately \$19 million.



#### PKU GOLIKE®



# Acquired U.S. rights to PKU GoLike, a medical formula for patients with phenylketonuria (PKU), in March 2024



**Controlled-release, taste- and odor-masked amino acid mix.** Designed to provide a better taste and a superior experience compared to alternative PKU medical formulas. Available in convenient bars and sachets.



**Ability to leverage existing metabolic sales force.** Product sold by the sales force that promotes Carglumic Acid, Betaine, and Nitisinone. Existing relationships in the metabolic community will significantly increase awareness and adoption.



**Strong interest since Q2 2024 launch.** Since launching the product under Eton's commercial infrastructure. Company's target is to reach 10% market share of the estimated \$100 million U.S. PKU medical food market.





#### Additional Metabolic Genetics Portfolio



#### Three approved generic products prescribed by metabolic geneticists



**Lower cost alternatives to very expensive rare disease products.** For some patients, the cost of Carbaglu® can exceed \$1 million per year.



**Promoted by Eton's sales force with full patient support offerings.** Prescriber base already covered by current commercial organization and patients have full access to Eton Cares and other programs.



**Strong financial contribution**. Acquisition of approved ANDAs has generated immediate revenue and high returns on invested capital. Expect metabolic genetics portfolio to contribute double digit million revenue annually.

#### Indication

**Eton Product Launch** 

Est. U.S. Patient Population

Est. Market Size



#### Hyperammonemia

Dec. 2021

~100

~\$50 Million



#### Betaine Anhydrous for oral solution

#### Homocystinuria

May 2023

1,000 - 2,000

\$10 -20 Million



## Nitisinone Capsules

#### Tyrosinemia Type 1

Q1 2024

200 - 300

~\$50 million

# **Under Development: ET-600**



#### Product candidate under development for the treatment of diabetes insipidus



**Addresses significant unmet need for an orphan patient population.** The condition is estimated to impact less than 5,000 pediatric patients in the United States.



**Synergistic fit in Eton's portfolio.** Product would be prescribed by pediatric endocrinologists, the same call point as ALKINDI SPRINKLE.



Proprietary, patent-pending product expected to have long-term patent protection.

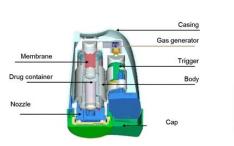


**Potential early 2025 NDA submission.** Passed pilot bioequivalence in Q1 2024, pivotal study initiated in Q4 2024.

### Under Development: ZENEO® Hydrocortisone Autoinjector



#### ZENEO® hydrocortisone autoinjector is under development as a rescue treatment for adrenal crisis





**Solves a major hurdle to current treatment.** Current treatment, Solu-Cortef®, must be diluted, mixed, and administered with a standard syringe. Eton's autoinjector would offer a ready-to-use product.





Solu-Cortef®



**Strong demand and market opportunity.** 200,000+ units of Solu-Cortef® are sold through retail or mail order annually. Total estimated market opportunity of \$100+ million annually\*.



**Demonstrated confidence in technology.** Development partner Crossject received a \$60 million order from the U.S. government for a different molecule that is utilizing the same device.

(Current standard of care)

# **Investment Summary**





# ATTRACTIVE PORTFOLIO AND PIPELINE POISED FOR LONG-TERM GROWTH

- 15 straight quarters of product revenue growth
- Expect continued organic growth from commercial portfolio
- Three late-stage rare disease products with potential nearterm launches (ET-400 PDUFA date February 2025)



# PROVEN ABILITY TO ACCELERATE GROWTH THROUGH ACQUISITIONS & LICENSING

- Favorable acquisition environment
- Actively considering opportunities to add additional products
- Announced acquisition of Increlex (Q4 2024)



# WELL-CAPITALIZED TO FUND GROWTH

- \$20.3 million of cash on hand\*
- Operating cash flow and net income positive in Q3 2024
- Significant excess cash to fund acquisitions



# NEAR-TERM CATALYSTS

- Closing of Increlex acquisition (late 2024)
- ET-600 pivotal study in Q4 2024
- ET-400 PDUFA date (Feb 28, 2025)
- Expected ET-600 NDA submission in Q1 2025

<sup>\*</sup> As of September 30, 2024



Appendix



©2024 Eton Pharmaceuticals. All rights reserved.

# **Experienced Leadership Team**



# Our leadership team brings a proven track record of successfully developing and commercializing products at industry leading companies



Sean Brynjelsen
Chief Executive Officer & Director



James Gruber, CPA Chief Financial Officer



David Krempa
Chief Business Officer



Scott Grossenbach

Vice President, Sales Operations



Danka Radosavljevic

Vice President, Quality



Kevin Guthrie

Executive Vice-President, Commercial
Operations















