



PORTOLA

PHARMACEUTICALS

Corporate Presentation

William Blair Annual Growth Conference

June 6, 2019

Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. In some cases you can identify these statements by forward-looking words, such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “intend,” “could,” “would,” “project,” “plan,” “potential,” “seek,” “expect,” “goal,” or the negative or plural of these words or similar expressions.

These forward-looking statements are subject to a number of risks, uncertainties and assumptions, and new risks emerge from time to time. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this presentation may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Please refer to our Annual Report on Form 10-K and our most recent Quarterly Report on Form 10-Q that we filed with the SEC for a description of risks and uncertainties that could impact future results.

Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur. We undertake no obligation to update any forward-looking statements except as required by law.

Momentum-Building Highlights

- Andexxa[®] Revenues Grew 45% Over Fourth Quarter to \$20.3 Million
- Ondexxya[®] Granted Conditional Marketing Authorization in Europe
- Andexxa U.S. Launch Off to a Great Start
- Key Data Presentations for Andexxa and Cerdulatinib in Q2





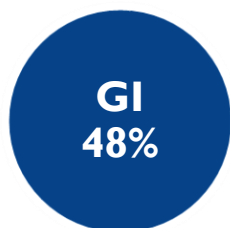
ANDEXXA

Factor Xa Inhibitor Bleeding – A Major and Growing Problem in the U.S.



140,000
U.S. hospital
admissions / year²

>80% of patients treated³



ICH Mortality & Morbidity

- 45-48% mortality at 30 days⁵
- 13 days in hospital⁶
- Top 20% with costs >\$100,000⁶

1. IMS U.S. Factor Xa Units. Oct. 2017 – Oct. 2018.

2. Truven Healthcare Analytics, a claims database with healthcare data for more than 43.6 million covered lives. Dec. 2017

3. Portola Market Research

4. Studies: Aristotle, Averroes, Rocket, Einstein & Engage-AF

5. Studies: Aristotle & Rocket

6. Truven

Andexxa – Addressing the Unmet Need

EFFECTIVE

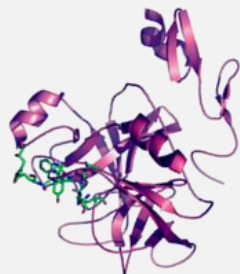
82%

Excellent/ Good
Hemostatic Efficacy¹

FAST

>90% reduction
of anti-Xa activity within just
2 MINUTES²

SPECIFIC



*Modified Factor Xa Decoy*³

SUPPORTED

- **First and only** antidote approved for patients treated with apixaban or rivaroxaban
- First-line recommendation in multiple guidelines



6
1. ANNEXA-4 (New England Journal of Medicine, Feb 2019)
2. Siegel, et al. *New England Journal of Medicine*. 2015.
3. Andexxa PI

Andexxa Demand in the U.S. is Strong and Growing



~100 additional
hospitals stocked
(Q4 YTD → Q1 YTD)

~200 → ~300



Increasing % of
hospitals with
reorders
(Q4 YTD → Q1 YTD)

50% → 55%



Four consecutive
quarters of
strong revenues

\$20.3 (Q1'19)

\$14.0 (Q4)

\$7.7 (Q3)

\$2.2 (Q2)

Effective Hemostasis at 12 Hours Post Andexanet

Number of Major Bleeds Adjudicated	Number of Patients who Achieved Excellent or Good Hemostasis	Percent of Patients who Achieved Excellent or Good Hemostasis	Binomial Exact 95% Confidence Interval
249	204*	82%	77% – 87%

****Of 204 patients, 171 (84%) were “excellent” and 33 (16%) were “good”***

Durability of Andexxa Response Maintained at 12 Hours

- 71 efficacy evaluable patients had non-traumatic, single-compartment, intraparenchymal hemorrhages
- Of these, 56 had volume expansion $\leq 35\%$ from baseline at 1 hour
- Of these, **55 of 56 (98%)** maintained excellent or good hemostasis at 12 hours

* Hematoma volume remained $\leq 35\%$ vs. baseline

Safety – Mortality and Thrombotic Events

Patients in Safety Analysis (N=352)	Total
Mortality within 30 days	49 (13.9%)
Patients with at least one thrombotic event within 30 days	34 (9.7%)



Safety – Restarting Anticoagulation

Thrombotic Events

34 (9.7%)



0

Before oral anticoagulation restart or never restarted

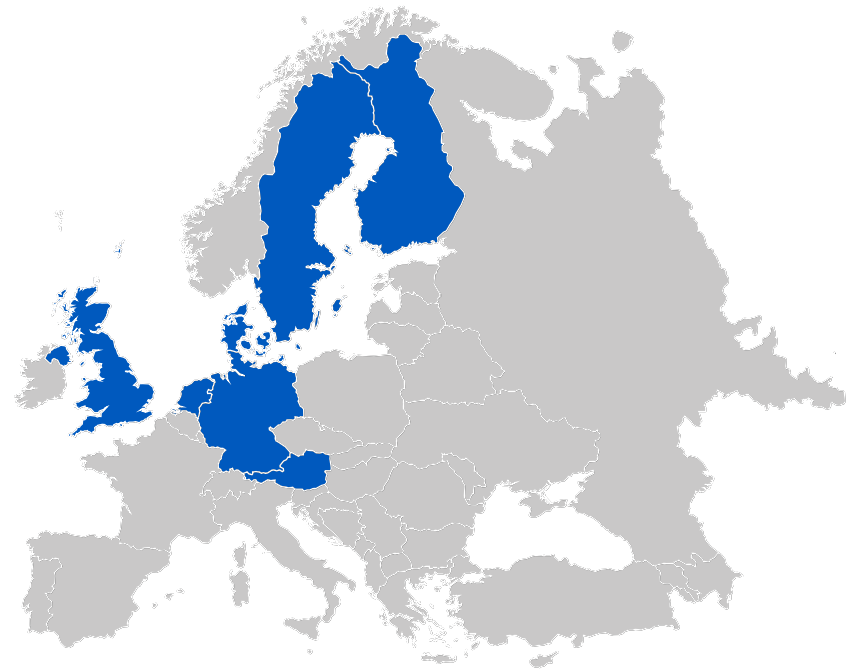
After oral anticoagulation restart

Patients in Safety Analysis (n=352)	Total
Restart of any anticoagulation (includes prophylactic dose heparins)	220 (62%)
Restart of oral anticoagulation	100 (28%)

Pursuing the Significant Opportunity in Europe

- European Commission approval on April 26, 2019
- Estimated # of eligible patients is equal to or greater than the U.S.
- Staged European launch in wave I countries
 - Germany, the U.K., Austria, the Netherlands and select Nordics
- Building out team with talented and tenured staff focused on education and awareness
- Reimbursement discussions underway
- Expect to report first European sales in late 2H 2019

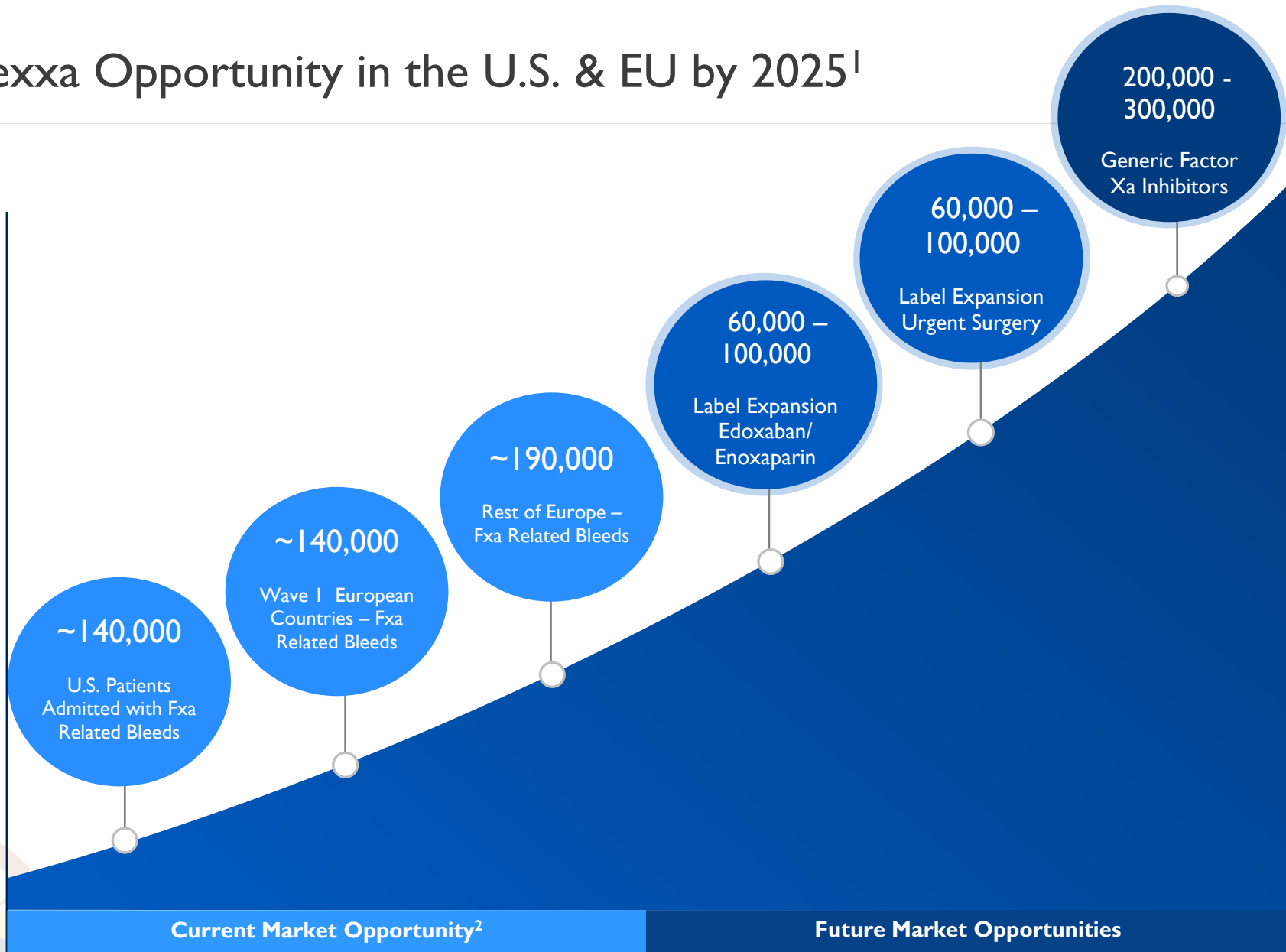
Ondexxya[®]
andexanet alfa



Simultaneously, continuing to evaluate partnerships in Europe and beyond

Andexxa Opportunity in the U.S. & EU by 2025¹

NUMBER OF PATIENTS



Current Market Opportunity²

Future Market Opportunities



CERDULATINIB



Cerdulatinib: Urgent Need for New Treatment Options for Relapsed/Refractory Patients with PTCL and CTCL

3,600 relapsed/refractory ¹



PTCL

5-Year Survival Rates ³

10% - 30% for most subtypes

2,000 relapsed/refractory ²



CTCL

ORR with Current Therapies

25% - 30% for R/R PTCL* & CTCL

**Exception is CD30+ ALCL given CD30-targeted treatment option (Adcetris)*

Significant unmet need for an oral agent that delivers higher response rates and a better safety profile

1. NIH SEER database – Cancer Stat Facts, 2018; Moskowitz et. al., *How I treat the peripheral T-cell lymphomas*. Blood. 2014 Apr 24; 123(17): 2636–2644
2. SEER Database; ; Epidemiology and Prognosis of T-Cell Lymphoma; S.S. Wang and J.M. Vose, *Oncology Journal*, August 2015
3. International T-cell Lymphoma Project. *Journal Clinical Oncology*, 2008; 26:4124-4130.

Phase 2a Response Summary in Target Populations



	PTCL		CTCL
	Overall	AITL (sub-type)	
N Evaluable	41	14	27
ORR	34%	57%	26%
CR	27%	50%	7%

- Received Orphan Drug Designation for PTCL from FDA in September 2018
- Productive end-of-phase II meeting with the FDA in January 2019
- FDA agreed that a single-arm study design is reasonable and would support accelerated approval
- Submitting additional data to support the proposed dose
- Pending the outcome of our discussions, we anticipate starting a registrational study by year-end

Advancing Cerdulatinib

- Submitted additional data requested by the FDA regarding the proposed dose for a registrational trial
- Presenting new data at ICML/Lugano and EHA this month evaluating cerdulatinib alone and in combination with rituximab for follicular lymphoma



Q1 2019 Financial Highlights (please see press release and 10-K for additional details)

- Total revenues of \$22.2 million led by Andexxa product net revenues of \$20.3 million
- Total operating expenses
 - GAAP operating expenses \$95.8 million
 - Non-GAAP operating expenses \$86.0 million*
- R&D expenses for the first quarter \$35.6 million
 - Decrease driven by Gen 2 manufacturing being capitalized into inventory
- SG&A expenses for the first quarter \$53.0 million
 - Increase driven by expanded sales force, commercial activity for Andexxa launch and launch preparations in Europe
- Cash balance of \$322.8 million at Mar. 31, 2019

*Please see the reconciliation of GAAP to non-GAAP financial measures included in today's quarterly press release which is posted in the Investor Relations section of our website.

PORTOLA

PHARMACEUTICALS

NASDAQ: PTLA

