PTC Therapeutics: EMFLAZA™ Acquisition Overview

March 16, 2017

Forward looking statements within the meaning of The Private Securities Litigation Reform Act of 1995

This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements, other than those of historical fact, contained in this release are forward-looking statements, including statements related to PTC's expectations with respect to the closing of its planned acquisition of all rights to Emflaza™ (deflazacort), or the "planned acquisition"; the potential financial impact and benefits to PTC of the planned acquisition, including with respect to a potential launch of Emflaza and PTC's expectations with respect to contingent payments to Marathon based on annual net sales; the potential advantages of Emflaza; the future expectations, plans and prospects for PTC; PTC's strategy, future operations, future financial position, future revenues or projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "look forward", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: satisfaction of the conditions to closing the acquisition (including the failure to obtain necessary regulatory approvals) in the anticipated timeframe or at all; PTC's ability to realize the anticipated benefits of the acquisition, including the possibility that the expected benefits from the planned acquisition will not be realized or will not be realized within the expected time period; negative effects of this announcement on the market price of PTC's common stock; significant transaction costs; unknown liabilities; the risk of litigation and/or regulatory actions related to the planned acquisition; other business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of Translarna™ (ataluren) and Emflaza; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q or Annual Report on Form 10-K as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna or Emflaza.

The forward-looking statements contained herein represent PTC's views only as of the date of this presentation and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this presentation except as required by law.

PTC acquiring Emflaza™ (deflazacort): Strong strategic fit

- PTC has been a pioneer in Duchenne muscular dystrophy R&D
  - Twenty year commitment to bring new therapies to DMD patients
  - $500 million of R&D investment; continued investment going forward
- Emflaza is the first treatment approved in the U.S. for use in all DMD patients, five years and older regardless of genetic mutation
- PTC is committed to enable access to Emflaza for DMD patients
- PTC to acquire all rights to Emflaza from Marathon Pharmaceuticals
- Total consideration: $140 million in cash and stock, plus sales-based contingent payments and a single milestone payment

Strategic rationale: Expect near-term U.S. revenues to support continued investment in DMD R&D

- Aligns with PTC's mission of bringing best-in-class therapies to patients with rare diseases
- Continues PTC's commitment to U.S. DMD patients and families
- Diversifies portfolio with second commercial product
- Establishes U.S. commercial footprint for potential Translarna™ launch if FDA approved
- Expected free cash flow supports continued R&D investment to discover and develop new treatments for rare disorders

Transaction Overview

- PTC to acquire all rights to Emflaza™ (deflazacort) from Marathon Pharmaceuticals on a cash free / debt free basis, subject to terms and conditions of the asset purchase agreement, as well as certain employees
- Upfront consideration: $140 million comprised of approximately $75 million in cash and approximately $65 million in PTC equity(1)
- Contingent consideration: payments based on Emflaza annual net sales beginning in 2018 which we expect will range as a percentage of annual net sales from the low to mid-20's, on a blended average basis as well as a single $50 million sales-based milestone
- Anticipate closing in Q2, subject to satisfaction of customary closing conditions including Hart-Scott-Rodino antitrust approval

(1) $65 million of PTC shares to be delivered at close based on 15 day VWAP share price ending on the 3rd trading day prior to close; subject to 6.9 million share limit. Any shortfall from $65 million value to be made whole with additional cash consideration.

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Overview of Emflaza™

- Emflaza™ is the first approved in the U.S. for use in all DMD patients, five years and older regardless of genetic mutation
- Received FDA approval on February 9, 2017; granted seven years orphan drug exclusivity
- By addressing inflammation, Emflaza is a critical component in Duchenne treatment to preserve muscle function and ultimately slow disease progression
- We believe that Emflaza is disease modifying and should be available for DMD patients as part of their totality of care

Emflaza™
(deflazacort)

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