



**Investor Conference Call:
Fourth Quarter and Full Year 2019 Financial Results
and Business Update**

February 13, 2020

On Today's Call



Prepared Remarks

- Michael G. Kauffman, MD, PhD, *Chief Executive Officer*
- Mike Mason, MBA, *Chief Financial Officer*



Joining for Q&A Session

- Perry Monaco, *Senior Vice President, Sales*
- Christopher Primiano, JD, MBA, *Chief Business Officer & General Counsel*
- Jatin Shah, MD, *Executive Vice President, Chief Medical Officer*
- Ian Karp, MBA, *Vice President, Investor and Public Relations*

Forward-looking Statements and Other Important Information

This presentation contains forward-looking statements within the meaning of the “safe harbor” provisions of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding Karyopharm’s expectations relating to XPOVIO® for the treatment of patients with heavily pretreated multiple myeloma, the therapeutic potential of and potential clinical development plans and commercialization for Karyopharm’s drug candidates, including the timing of initiation of certain trials, of the reporting of data from such trials, of the submissions to regulatory authorities and of potential commercial launches; the potential availability of accelerated approval pathways; the potential size of the markets for multiple myeloma drugs and multiple myeloma drugs for treatment of patients with relapsed multiple myeloma; the potential size of the markets for diffuse large B-cell lymphoma (DLBCL) drugs and DLBCL drugs for treatment of patients with relapsed and / or refractory DLBCL; and Karyopharm’s strategic and financial plans and expectations as well as financial projections for Karyopharm. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from Karyopharm’s current expectations. For example, there can be no guarantee that Karyopharm will successfully commercialize XPOVIO, that regulators will agree that selinexor qualifies for conditional approval in the E.U. as a result of the data from the STORM study in patients with penta-refractory myeloma or accelerated approval in the U.S. based on the SADAL study in patients with relapsed/refractory DLBCL or that any of Karyopharm’s drug candidates, including selinexor and eltanexor (KPT-8602), Karyopharm’s second generation SINE compound, or KPT-9274, Karyopharm’s first-in-class oral dual inhibitor of PAK4 and NAMPT, or any other drug candidate Karyopharm is developing, will successfully complete necessary preclinical and clinical development phases or that development of any of Karyopharm’s drug candidates will continue. Further, there can be no guarantee that any positive developments in the development or commercialization of Karyopharm’s drug candidate portfolio will result in stock price appreciation. In addition, even if Karyopharm receives marketing approval for selinexor in additional indications or for any other drug candidate, there can be no assurance that Karyopharm will be able to successfully commercialize that drug candidate. Management’s expectations and, therefore, any forward-looking statements in this presentation could also be affected by risks and uncertainties relating to a number of other factors, many of which are beyond Karyopharm’s control, including the following: adoption of XPOVIO in the commercial marketplace; the timing and costs involved in commercializing XPOVIO or any of Karyopharm’s drug candidates that receive regulatory approval; the ability to retain regulatory approval of XPOVIO or any of Karyopharm’s drug candidates that receive regulatory approval; Karyopharm’s results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the FDA and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies, including with respect to the need for additional clinical studies; the ability of Karyopharm or its third party collaborators or successors in interest to fully perform their respective obligations under the applicable agreement and the potential future financial implications of such agreement; Karyopharm’s ability to obtain and maintain requisite regulatory approvals and to enroll patients in its clinical trials; unplanned cash requirements and expenditures; development of drug candidates by Karyopharm’s competitors for diseases for which Karyopharm is currently developing its drug candidates; that the markets for multiple myeloma and DLBCL drugs will grow as predicted; and Karyopharm’s ability to obtain, maintain and enforce patent and other intellectual property protection for any drug candidates it is developing. These and other risks are described under the caption “Risk Factors” in Karyopharm’s Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, which was filed with the Securities and Exchange Commission (SEC) on November 4, 2019, and in other filings that Karyopharm may make with the SEC in the future. Any forward-looking statements contained in this presentation are for informational purposes only and speak only as of the date hereof. Other than as is required by law, Karyopharm expressly disclaims any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise. Karyopharm’s website is <http://www.karyopharm.com>. Karyopharm regularly uses its website to post information regarding its business, drug development programs and governance. Karyopharm encourages investors to use www.karyopharm.com, particularly the information in the section entitled “Investors,” as a source of information about Karyopharm. References to www.karyopharm.com in this presentation are not intended to, nor shall they be deemed to, incorporate information on www.karyopharm.com into this presentation by reference. Unless otherwise noted, this presentation contains data that are interim and unaudited based on site reports. In addition, data included in this presentation have not been updated and are as of the cutoff date for the applicable medical conference presentation. Other than the accelerated approval of XPOVIO, selinexor, eltanexor, KPT-9274 and verdinexor are investigational drugs that have not been approved by the FDA or any other regulatory agency, and the safety and efficacy of these drugs has not been established by any agency.

2019 was a Landmark Year for Karyopharm with a Strong Fourth Quarter Finish



Commercial Update

- **July 3rd** FDA accelerated approval of XPOVIO
- Q4 2019 XPOVIO sales of **\$17.7M**
- Full year 2019 XPOVIO sales of **\$30.5M**
- **>550** physicians / accounts prescribed XPOVIO in first 6 months



Pipeline / Clinical Data Update

- BOSTON Phase 3 top-line data on-track, expected **early 2020**
- **22** presentations at ASH 2019
- DLBCL sNDA submitted **Dec 2019**
- MAA decision in Europe expected **mid-2020**
- **5 new** clinical trials expected to start in 2020

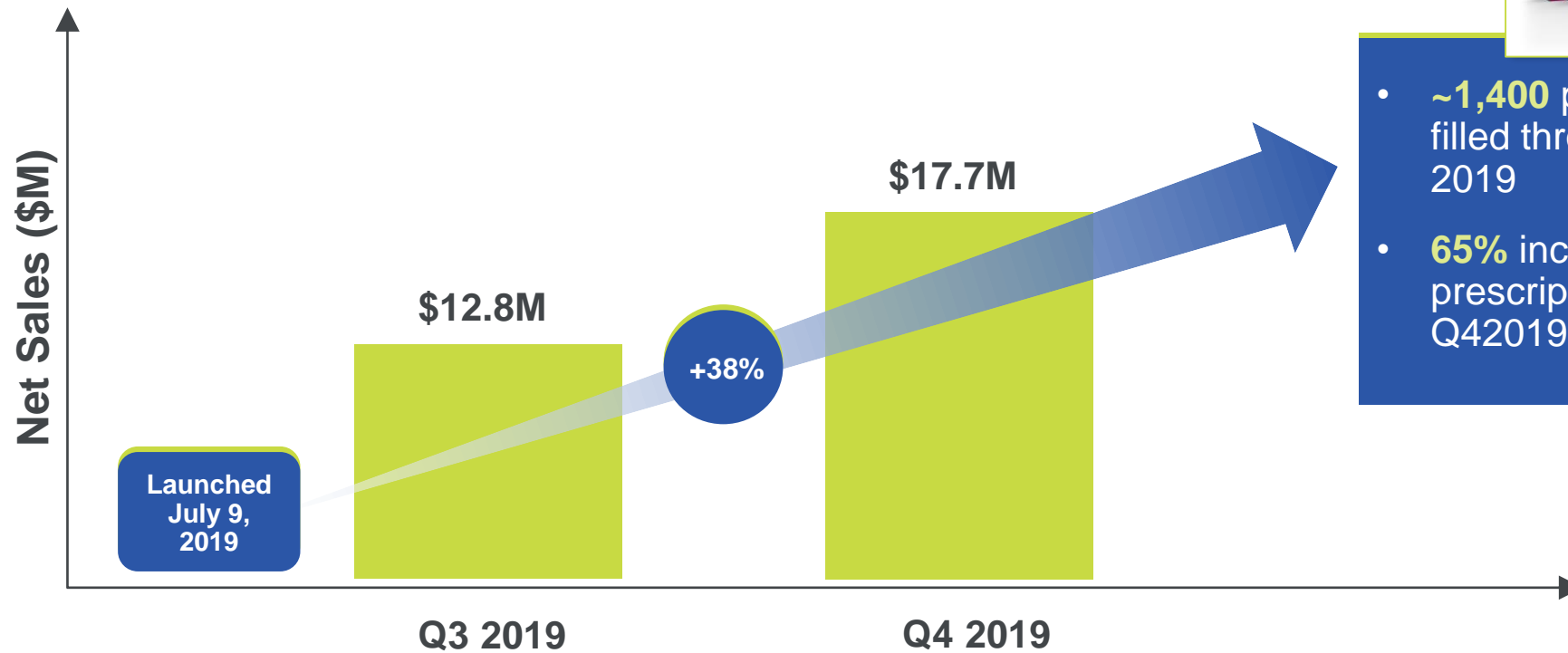


Balance Sheet

- Ended Q4 2019 with **~\$266 million** in cash, cash equivalents, restricted cash and investments
- Cash runway expected to be sufficient to fund planned operations into **middle of 2021**

Strong XPOVIO Sales Growth Following FDA Approval

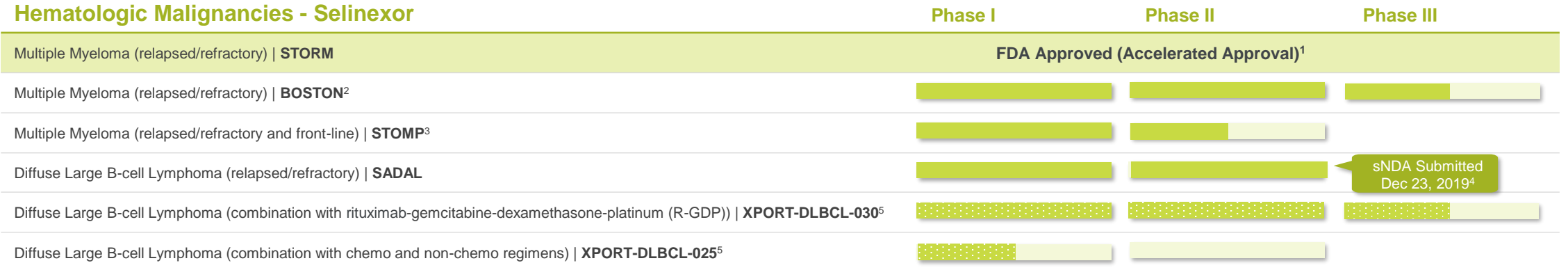
XPOVIO Product Sales Following Launch



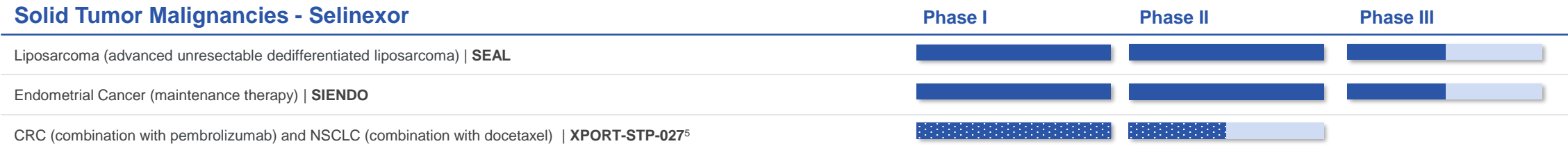
- **~1,400** prescriptions filled through Dec 31, 2019
- **65%** increase in prescription demand Q42019 vs. Q32019

Karyopharm's All Oral Clinical Pipeline

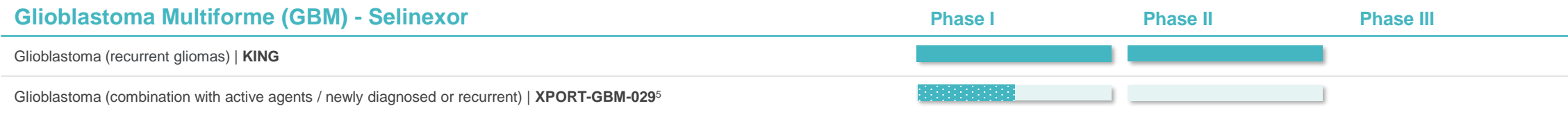
Hematologic Malignancies - Selinexor



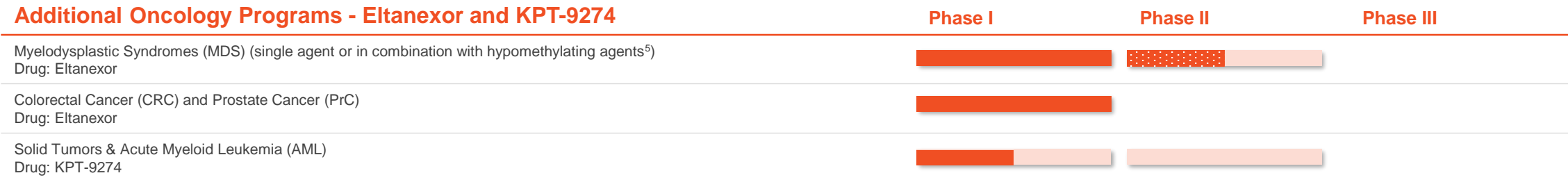
Solid Tumor Malignancies - Selinexor



Glioblastoma Multiforme (GBM) - Selinexor



Additional Oncology Programs - Eltanexor and KPT-9274



¹ Full Prescribing Information and Medication Guide are available at www.XPOVIO.com ² Oral selinexor, Velcade® (bortezomib) and dexamethasone vs. Velcade and dexamethasone. ³ Oral selinexor and dexamethasone + Revlimid® (lenalidomide), Pomalyst® (pomalidomide), Velcade, Kyprolis® (carfilzomib) or Darzalex® (daratumumab). ⁴ With request for accelerated approval (U.S.). ⁵ Study expected to start in 2020.



Fourth Quarter and Full Year 2019 Financial Results

Mike Mason
Chief Financial Officer



4th Quarter and Full Year 2019 Financial Results

Statement of Operations	Three Months Ended December 31 st		Twelve Months Ended December 31 st	
	2019	2018	2019	2018
Total Revenue	\$18.1M	\$0.2M	\$40.9M	\$30.3M
XPOVIO Net Sales	\$17.7M	---	\$30.5M	----
License and other Revenue	\$0.4M	\$0.2M	\$10.4M	\$30.3M
Total Operating Expenses	\$61.4M	\$57.7M	\$230.2M	\$210.2M
Cost of Sales	\$1.4M	----	\$2.4M	----
Research and Development Expense	\$31.6M	\$38.9M	\$122.3M	\$161.4M
Selling, General & Administrative Expense	\$28.4M	\$18.8M	\$105.4M	\$48.8M
Net Loss	\$48.6M (\$0.76 per share)	\$58.2M (\$0.96 per share)	\$199.6M (\$3.22 per share)	\$178.4M (\$3.14 per share)

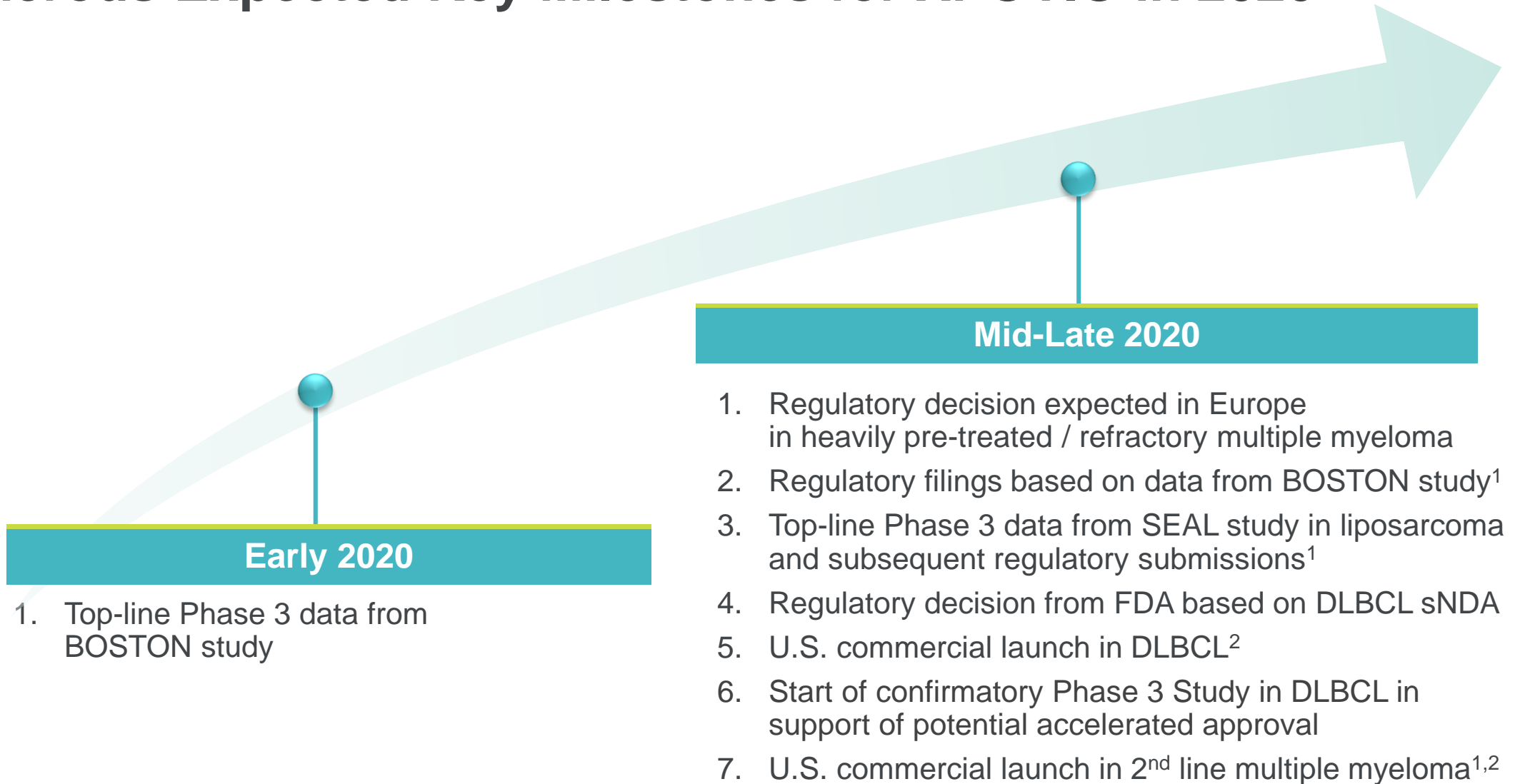
Balance Sheet and Financial Guidance

Balance Sheet	December 31, 2019	December 31, 2018
Cash, Cash Equivalents, Restricted Cash and Investments	\$265.8M	\$330.9M

- **Non-GAAP operating expense is expected to be \$240-260M for the full year 2020¹**
 - **Cash runway expected to be sufficient to fund planned operations into the middle of 2021**

¹ Excludes stock-based compensation expense. This outlook can only be provided on a non-GAAP basis because Karyopharm cannot reliably predict without unreasonable efforts the timing or amount of the factors that substantially contribute to the projection of stock compensation expense, which is excluded from the full year 2019 outlook for non-GAAP operating expenses.

Numerous Expected Key Milestones for XPOVIO in 2020



¹ Subject to positive Phase 3 results. ² Subject to regulatory approval.



Questions?

Answers.