



Corporate Presentation

JANUARY 2024

Forward Looking Statements

Forward Looking Statements

This presentation contains "forward-looking statements," as that term is defined under the Private Securities Litigation Reform Act of 1995 (PSLRA), which statements may be identified by words such as "expects," "plans," "projects," "will," "may," "anticipates," "believes," "should," "intends," "estimates," and other words of similar meaning, including statements regarding expected financial performance and expectations regarding the market for and sales of our products, whether ModeX will receive regulatory approval for products in development and be able to successfully commercialize products in its pipeline, whether we will initiate our planned clinical trials and whether our clinical trials will be successful, whether expectations regarding the benefits of and market for NGENLATM and Rayaldee will be met, the risk of downturns and a changing regulatory landscape in the highly competitive healthcare industry, whether our products will launch in all the territories in which they have been approved for sale, our product development efforts and the expected benefits of our products, whether our products in development will be commercialized, whether the relationships and collaborations with our business partners will be successful, whether our business partners will be able to commercialize our products and successfully utilize our technologies, whether our cost reduction efforts will be successful and we will be able to successfully grow operations in our diagnostics business, whether our sale of assets in the diagnostics segment will be consummated and yield anticipated results, whether the remaining business will be successful, our ability to market and sell any of our products in development, as well as other non-historical statements about our expectations, beliefs or intentions regarding our business, technologies and products, financial condition, strategies or prospects. Many factors could cause our actual activities or results to differ materially from the activities and results anticipated in forward-looking statements. These factors include those described in our Annual Reports on Form 10-K filed and to be filed with the Securities and Exchange Commission and under the heading "Risk Factors" in our other filings with the Securities and Exchange Commission, as well as the continuation and success of our relationship with our commercial partners, liquidity issues and the risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments. In addition, forward-looking statements may also be adversely affected by general market factors, competitive product development, product availability, federal and state regulations and legislation, the regulatory process for new products and indications, manufacturing issues that may arise, patent positions and litigation, among other factors. The forward-looking statements contained in this press release speak only as of the date the statements were made, and we do not undertake any obligation to update forward-looking statements. We intend that all forward-looking statements be subject to the safe-harbor provisions of the PSLRA.

Industry and Market Data

Industry and market data used in this investor presentation have been obtained from third party industry publications and sources, including reports by market research firms. The Company has not independently verified the information and data obtained from these sources and cannot assure you of the data's accuracy or completeness. This information and data is subject to change.

Our vision



ACCOMPLISHED
MANAGEMENT
TEAM WITH LONG
TRACK RECORD



INDUSTRY
LEADING
PARTNERS



ESTABLISHED
GLOBAL
MARKETS
WITH UNMET
NEED



NEXT-
GENERATION
PRODUCTS



PROVEN
TECHNOLOGIES

Business transformation continues to unleash OPKO's potential

FDA approval positions NGENLA™ as the leading long-acting growth hormone product partnered with Pfizer and now approved in more than 44 markets worldwide

Growth of multispecific antibody pipeline through advancement and partnering creates new opportunities in I/O and infectious disease

Continued focus on improving profitability at BioReference through strategic revenue growth in high-value segments and expense reduction

Stable growth from operating businesses enables ongoing R&D commitment

Building a leading portfolio of next-generation therapies in fast-growing segments

GROWING COMMERCIAL HGH FRANCHISE

NGENLA™ long-acting efficacy to reshape the market

Estimated \$5.5 billion market expected to expand by up to 12.3% annually between 2023-2030¹

Proudly partnered with Pfizer



VALUE-CREATION ENGINE

Creating first-in-class multispecific antibodies to redefine what's possible in I/O and infectious disease

Robust pipeline of I/O and infectious disease programs

Proudly partnered with BARDA, Merck and NIH



CONTINUED FOCUS ON BIOREFERENCE PROFITABILITY

41% improvement of operating loss from Q2 to Q3 2023

Strategic growth through leadership in high-value specialty testing segments

DIVERSE GLOBAL PORTFOLIO

Stable growth by meeting important and growing patient needs

1. "Human Growth Hormone Market Size," Grand View Research, Grand View Research, <https://www.grandviewresearch.com/industry-analysis/human-growth-hormone-drugs-market>

Leadership team with unprecedented experience



Phil Frost
CEO and Chairman

Former Chairman and
Vice Chairman of Teva

Former Chairman and
CEO of IVAX



Jane Hsiao
Chief Technical
Officer and Vice
Chairman

Former Vice Chairman-
Technical Affairs of IVAX

Former Chairman, CEO
and President of IVAX
Animal Health



Elias Zerhouni
President and Vice
Chairman

Former President of
Global R&D of Sanofi

Former Director of NIH

Former Senior Fellow at
Bill and Melinda Gates
Foundation



Gary Nabel
Chief Innovation
Officer and Director

Former Chief Scientific
Officer at Sanofi

Founding Director of
NIH's Vaccine Research
Center



John Mascola
Chief Scientific
Officer, ModeX

Former Director of NIH's
Vaccine Research Center

Former leader in HHS's
"Operation WARP Speed"
COVID-19 effort



Addressing Established Markets with Next-Generation Solutions

DISEASE AREA

HUMAN GROWTH HORMONE DEFICIENCY

SOLID TUMOR CANCERS

LEUKEMIA AND LYMPHOMAS

INFECTIOUS DISEASES

MARKET SIZE

\$5.5B

GLOBAL MARKET¹

980,000+

ANNUAL U.S. CASES²

140,000+

ANNUAL U.S. CASES²

95%

OF GLOBAL POPULATION INFECTED WITH EBV ALONE³

UNMET NEED

NGENLA™ offers potentially better adherence through once weekly treatment

Multispecific treatments offer potential for deeper and more sustained responses to common cancers, broadening I/O's potential

Multitargeted approaches offer potential for both treatment and prevention of world's most urgent viral threats

¹ "Human Growth Hormone Market Size," Grand View Research, Grand View Research, <https://www.grandviewresearch.com/industry-analysis/human-growth-hormone-drugs-market>

² "Common Cancer Types", National Cancer Institute, National Cancer Institute, <https://www.cancer.gov/types/common/cancers>

³ "Epstein-Barr Virus," Cleveland Clinic, Cleveland Clinic, <https://my.clevelandclinic.org/health/diseases/23459-epstein-barr-virus>

Expanding OPKO's global footprint through partnerships



INDUSTRY- LEADING PARTNERS

Track record of partnering with world-leading organizations on attractive terms

SOUGHT OUT SCIENCE

OPKO's scientific expertise and leading multispecific position continues to offer value

RISK- AND COST-SHARING

Agreements allow OPKO to offset development risks and outsource sales & marketing costs

THINKING GLOBALLY

Continuing to seek partners with global commercial capabilities to reach largest number of patients

Preparing for global rollouts and offsetting R&D costs with industry-leading partners



NGENLA™

ELIGIBLE FOR ADDITIONAL

\$100M

IN MILESTONE PAYMENTS FOR
ADDITIONAL INDICATIONS AND GROSS
PROFIT SHARE ON GLOBAL SALES

\$90M

PAYMENT IN 2023



EBV NANOPARTICLE
VACCINE

ELIGIBLE FOR UP TO

\$872.5M

IN TOTAL MILESTONE PAYMENTS PLUS
ROYALTIES ON GLOBAL SALES

\$50M

PAYMENT IN 2023



COVID-19
MULTISPECIFIC

ELIGIBLE FOR UP TO

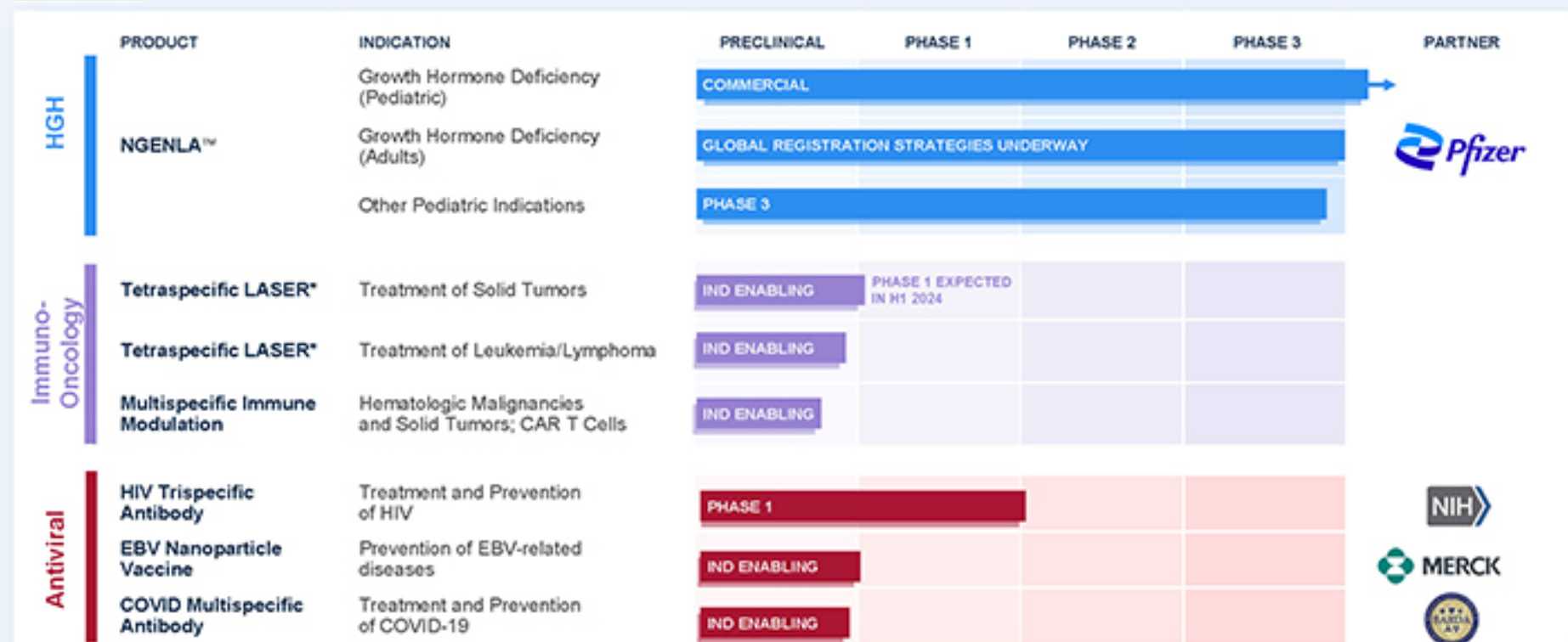
\$168M

IN TOTAL MILESTONE PAYMENTS
FOR OTHER VIRAL PROGRAMS

\$59M

COMMITTED IN 2023

Robust pipeline for well-understood diseases in need of next-generation treatments



*Lymphocyte Activator and Survival Extension Receptor (LASER) Antibodies

Human Growth Hormone Franchise

Focus on fast-growing markets with differentiated next-generation therapies

WE BELIEVE NGENLA™ IS POSITIONED TO GAIN SIGNIFICANT U.S. AND WORLDWIDE SHARE

**BETTER
ADHERENCE**

Market to consolidate
from 7 daily treatments
to 3 long-acting
therapies

**GROWING
GLOBAL
MARKET**

Estimated \$5.5 billion market expected to expand by up to 12.3% annually due to greater convenience and better adherence to weekly treatment¹

**NEXT-GEN
INNOVATION
FOR CHILDREN**

NGENLA™ approved
in over 40 markets
for the 1 in 3,500-
10,000 pediatric
patients affected by
GHD²

PROUDLY
PARTNERED
WITH PFIZER



Pfizer pursuing additional approvals for other indications

1. "Human Growth Hormone Market Size," Grand View Research, Grand View Research, <https://www.grandviewresearch.com/industry-analysis/human-growth-hormone-drugs-market>

2. "Growth hormone deficiency or rather short stature unresponsive to stimulation tests?" NH, NH, <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC608715/#:~:link=C%26deficiency%20of%20growth%20hormone%20use%20for%20the%20diagnosis>

Next-generation NGENLA™ poised to capture significant share of growing global market

CURRENT MARKET

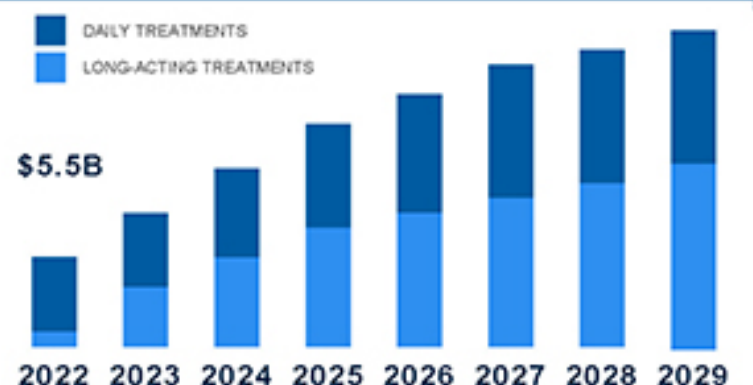
\$5.5B
Estimated global
hgH market in 2022¹

EXPANDING SHARE OF GROWING MARKET

TODAY

FUTURE

Market expected to grow by up to 12.3% annually due to greater convenience and better adherence to weekly treatment¹



NGENLA™ is positioned for significant worldwide share as one of 3 long-acting therapies replacing 7 daily treatments

7 DAILY TREATMENTS

3 LONG-ACTING TREATMENTS INCLUDING NGENLA™

¹ "Human Growth Hormone Market Size," Grand View Research, Grand View Research, <https://www.grandviewresearch.com/industry-analysis/human-growth-hormone-drugs-market>
² Accordis Pharma estimate

Continued upside from Pfizer partnership in 2024 and beyond



INDICATION

STATUS

Growth Hormone
Deficiency (Pediatric)

COMMERCIAL

Growth Hormone
Deficiency (Adults)

GLOBAL REGISTRATION STRATEGIES
UNDERWAY

Other pediatric
indications

PHASE 3

U.S. and European approval of adult
indication would unlock additional

\$50M IN MILESTONE
PAYMENTS

Approval of additional pediatric
indications would unlock additional

\$50M IN MILESTONE
PAYMENTS

Innovation Pipeline

Proprietary approaches with broad disease applicability

IMMUNO-ONCOLOGY



Multispecific antibodies

INFECTIOUS DISEASE



Multispecific antibodies



Multivalent nanoparticle vaccines

Strong IP around core technologies

Broad optionality in diseases not addressable by older modalities

Strong synergies across programs via shared technologies

Addressing large and critical patient needs in oncology with therapies that attack multiple disease drivers

NEXT-GENERATION THERAPIES



Multispecific antibodies designed to bind to two T cell antigens and two tumor antigens

PROVEN TECHNOLOGIES



Builds on the advances made by existing I/O antibody treatments but offers even greater specificity

Offers the potential to overcome resistance through tumor loss of a single antigen, a major cause of relapse

ESTABLISHED GLOBAL MARKETS WITH UNMET NEED

SOLID TUMORS¹

\$ 185B

300k

BREAST

288k

PROSTATE

238k

LUNG

153k

COLORECTAL

ANNUAL U.S. CANCER CASES⁴

BLOOD CANCERS^{2,3}

\$ 25B

140k+

NEW CASES

43k+

DEATHS

ANNUAL U.S. B CELL
LEUKEMIAS AND NON-HODGKIN
LYMPHOMAS⁴

1. "Solid Tumor Cancer Treatment Market," Precedence Research, Precedence Research, Dec. 2022, <https://www.precedenceresearch.com/solid-tumor-cancer-treatment-market>, Represents estimated market size as of 2022
2. "Leukemia Therapeutics Market by Type of Leukemia," Next Move Strategy Consulting, Next Move Strategy Consulting, Nov. 2023, <https://www.nextmoveconsulting.com/leukemia-therapeutics-market>, Represents estimated market size as of 2023
3. "Global Non-Hodgkin Lymphoma Therapeutics Market Size," Spheral Insights, Spheral Insights, Aug 2023, <https://www.spheralinsights.com/non-hodgkin-lymphoma-therapeutics-market>, Represents estimated market size as of 2023
4. "Common Cancer Types", National Cancer Institute, National Cancer Institute, <https://www.cancer.gov/types/commoncancers>

Tetraspecific LASER Antibodies for Immuno-Oncology

LUNG, PROSTATE, BREAST, UTERINE, COLON AND OTHER SOLID CANCERS

Lead candidate with IND submission expected in First Half 2024

Dual T-cell signaling stimulates T cells via CD3 and enhances survival via CD28, optimizing sustained T-cell killing of cancer cells



B CELL MALIGNANCIES: LYMPHOMA/LEUKEMIA

In IND-enabling phase

Dual T-cell signaling stimulates T cells via CD3 and enhances survival via CD28, optimizing sustained T cell killing of cancer cells



Tackling EBV, one of the world's most common viruses with no approved vaccines or therapies

NEXT-GENERATION THERAPIES



Nanoparticle EBV candidate targeting four viral proteins

PROVEN TECHNOLOGIES



Next-generation vaccine for cancer prevention

1. "Epstein-Barr Virus," Cleveland Clinic, Cleveland Clinic, <https://my.clevelandclinic.org/health/diseases/23409-epstein-barr-virus>
2. Khan G, Fitzmaurice C, Naghavi M, et al. "Global and regional incidence, mortality and disability-adjusted life-years for Epstein-Barr virus-attributable malignancies, 1990–2017" BMJ Open 2020;10:e007505. doi: 10.1136/bmjopen-2020-007505

ESTABLISHED GLOBAL MARKETS WITH UNMET NEED



95% OF GLOBAL POPULATION INFECTED BY ADULTHOOD ¹

265,000

ESTIMATED LINKED ANNUAL CANCER CASES ²

163,000

ESTIMATED LINKED ANNUAL CANCER DEATHS ²



NO APPROVED THERAPIES OR VACCINES



Improving treatment of infectious disease through multispecifics that address viral evolution

NEXT-GENERATION THERAPIES



Multispecific long-acting antibodies platform allows for the rational selection of multiple targets designed to overcome drug resistance and prevent viral escape

PROVEN TECHNOLOGIES



Harnesses antibodies for treatment of infectious disease

Could potentially be used for acute and preventative treatment

ESTABLISHED GLOBAL MARKETS WITH UNMET NEED



COVID-19



HIV

EARLY RESEARCH FULLY FUNDED THROUGH BARD PARTNERSHIP

PHASE 1 TRIAL SPONSORED AND FUNDED BY NIH



CURRENT TREATMENTS

Lack efficacy against new SARS-CoV-2 strains

Lead to toxicity and drug resistance



POTENTIAL FOR TREATMENT

Antibodies that remain potent as both treatment and prevention



OPPORTUNITY FOR MULTISPECIFICS

Antibodies that retain potency against new variants

Diagnostics Division

Continued focus on improving profitability through strategic growth and cost reduction

SPECIALTY TESTING SEGMENTS OFFER STRATEGIC GROWTH

Oncology

Women's Health

Hospital & Health
Systems

Urology

IMPROVING PRODUCTIVITY AND ENHANCING INNOVATION IN HIGH-VALUE SPECIALTY TESTING SEGMENTS

BEST-IN-CLASS ONCOLOGY TESTING PORTFOLIO

Launching cutting-edge homologous recombination deficiency test to provide insights on genomic instability

Potential to be the test that guides patient eligibility for PARP inhibitors

Set to launch expanded hematological malignancy panel

EXPANDING SERVICE LINES THROUGH EXISTING CAPABILITIES

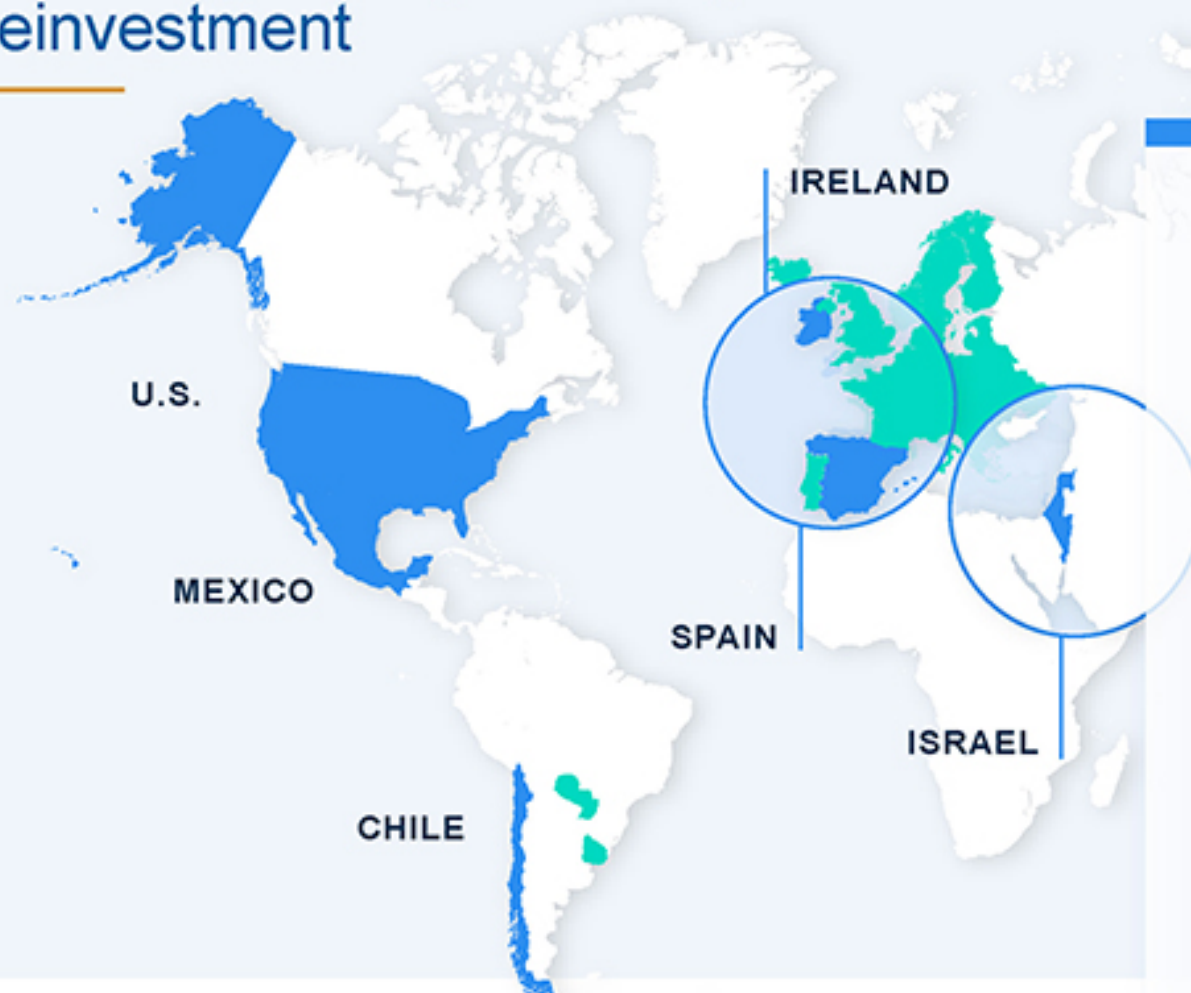
Offering access to our high-value data and analytics for pharmaceutical companies and CROs

+41%

**IMPROVEMENT
OF OPERATING LOSS
FROM
Q2 TO Q3 2023**

Operating Businesses

Operating units generating additional income for reinvestment



GLOBAL FINANCIALS THROUGH Q3 '23

\$ 681.6M

TOTAL SALES

\$ 21.7M

RAYALDEE SALES

TOTAL SALES BY COUNTRY

U.S.: \$463.0M	Spain: \$16.4M
Ireland: \$121.2M	Israel: \$9.4M
Chile: \$52.4M	Other: \$1.7M
Mexico: \$17.4M	

Key Takeaways

Transformative 2023 serves as springboard for further growth in 2024

2023 ACCOMPLISHMENTS

- ✓ NGENLA™ receives U.S. FDA approval
- ✓ New partnership agreements signed with over \$1 billion in potential milestone payments
- ✓ Continued progress on creating efficiencies and improving profitability in diagnostics segment
- ✓ ModeX fully integrated into OPKO Health