

DNB Small & Medium Enterprises

April 5th 2017

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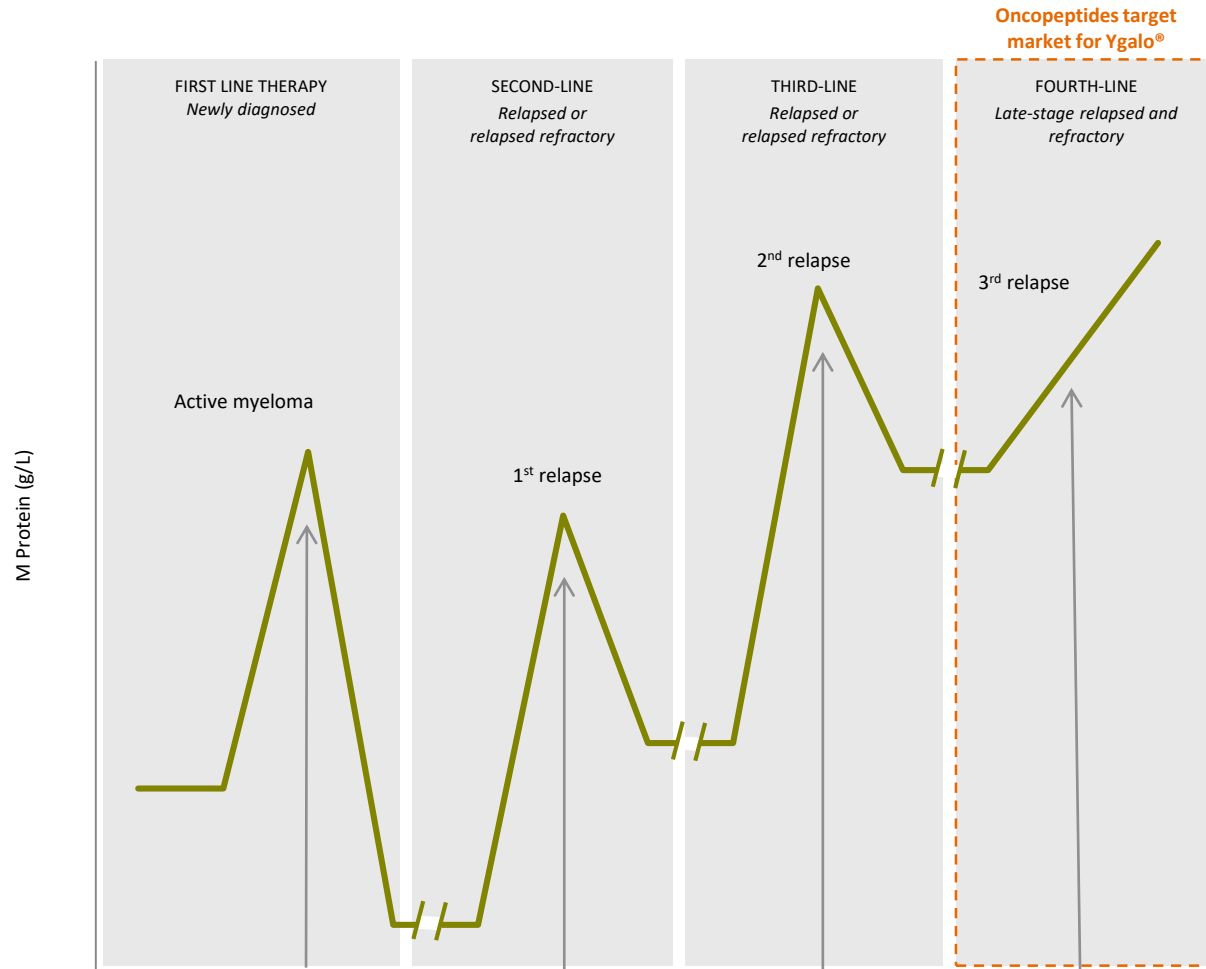
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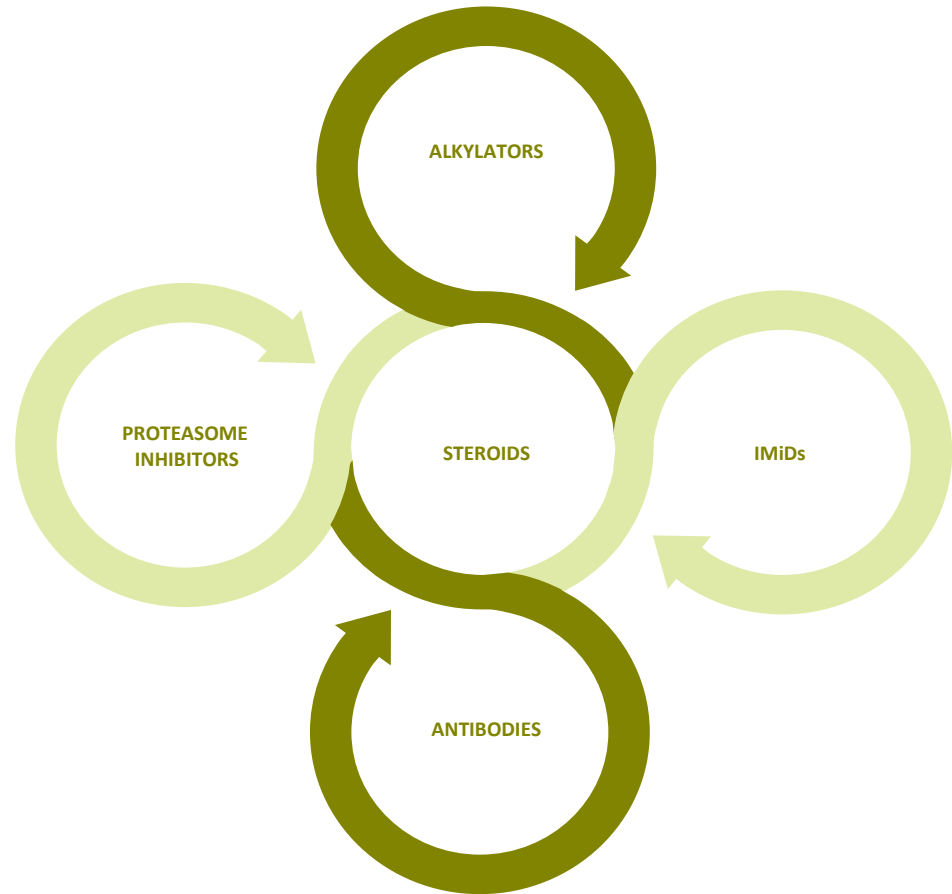
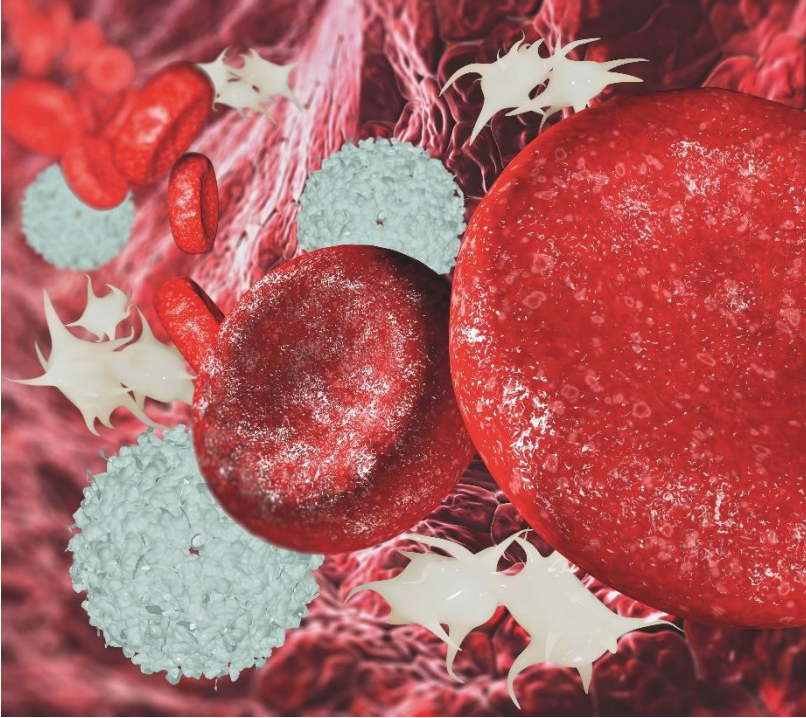
Summary

- FULLY FUNDED DEVELOPMENT PROGRAM IN MYELOMA INCLUDING PHASE III – READ-OUT IN 2019
- EFFICACY DATA SUPPORTS SUPERIORITY OVER STANDARD OF CARE IN LATE-STAGE MULTIPLE MYELOMA – POTENTIAL of 1.3+bn USD AND 30% GROWTH
- OUR CANDIDATE YGALO® HAS ORPHAN DRUG DESIGNATION IN THE US AND EU
- FULLY AGREED CLINICAL PROGRAM WITH THE FDA AND EU AUTHORITIES
- MULTIPLE PATHS TO APPROVAL TO DE-RISK DEVELOPMENT PROGRAM

Multiple myeloma is a hematologic cancer with no cure – all patients become resistant to treatment and relapse into disease progression

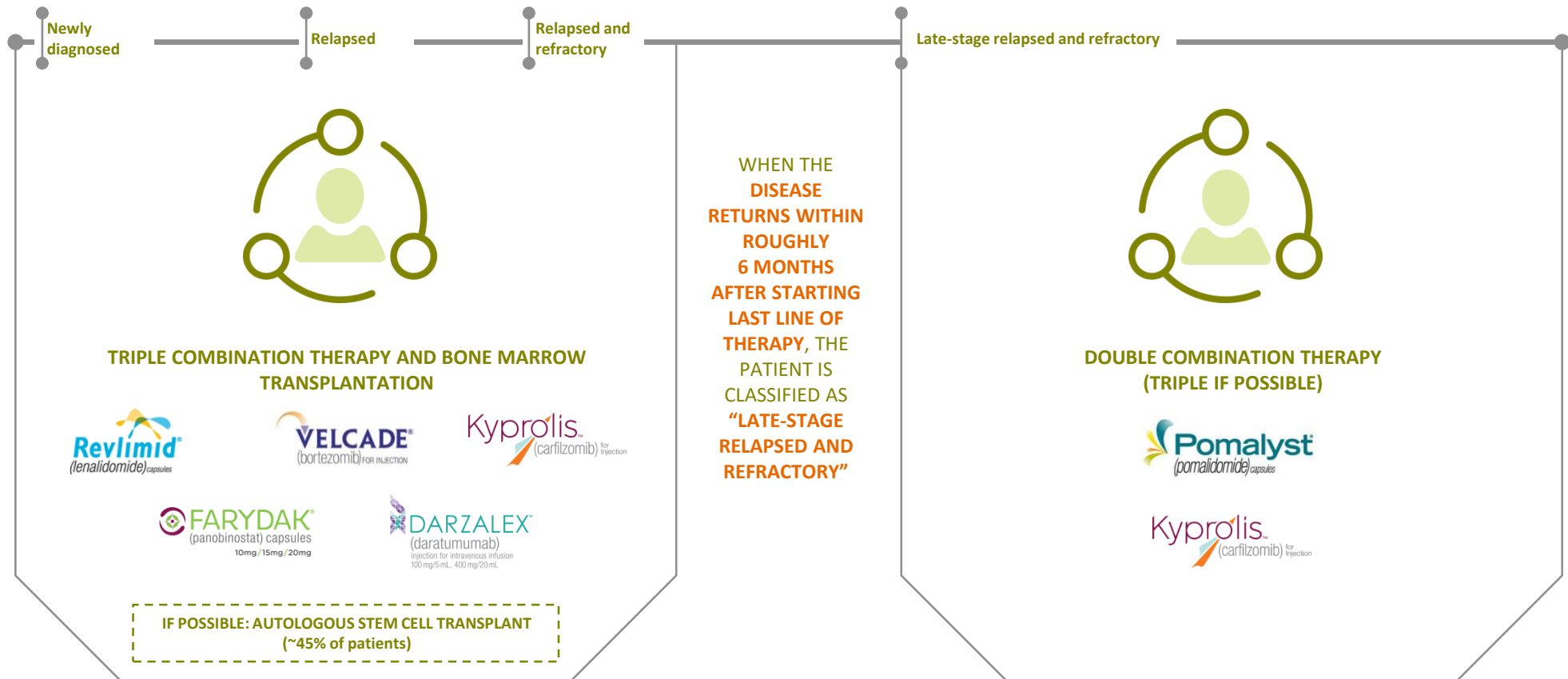


Multiple myeloma is mainly treated with broad spectrum cytotoxic agents with significant off-target effects



Limited number of treatment options for late-stage RRMM patients despite advances in treatment of early-stage MM

Lines of therapy throughout the disease stages¹⁾



Limited number of treatment options for late-stage RRMM patients –
Novel treatment options are necessary and demanded by patients and regulatory bodies

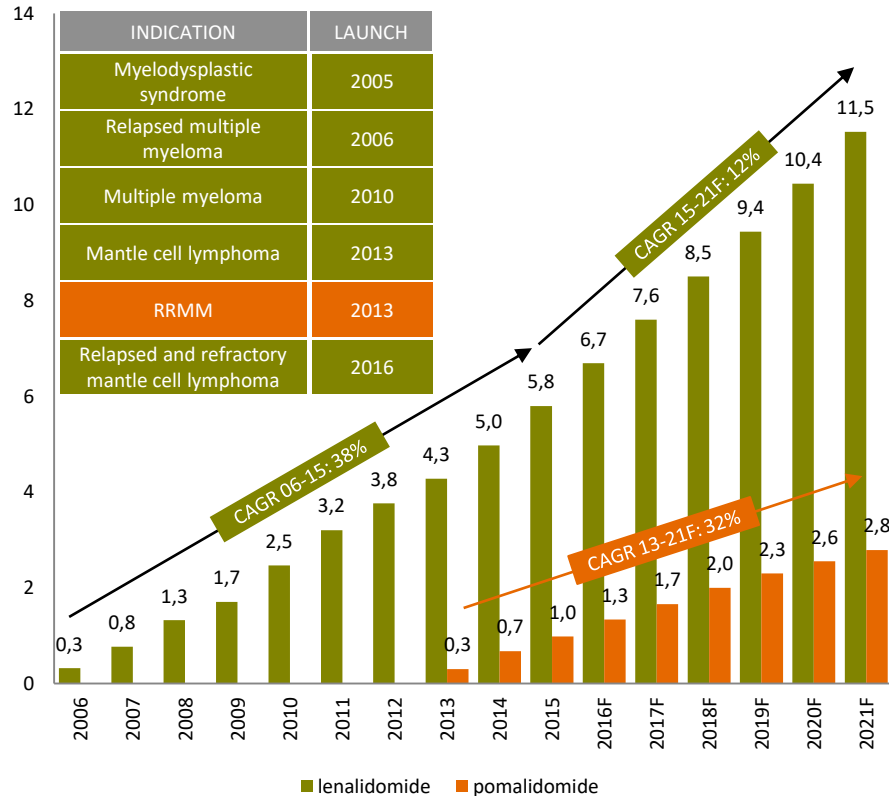
Source: GlobalData. Steroids excluded (almost 100% patient share)

1) Including those with an estimated market share above 5% in respective stages in the US during 2016

Current standard of care across stages in MM are two blockbuster products marketed by Celgene – 8+bn USD in sales and double digit growth

Blockbuster products with rapid uptake post launch¹⁾

USDbn



BRANDS (SELECTION)

Revlimid®
(lenalidomide) capsules

Pomalyst®
(pomalidomide) capsules

Pomalidomide was developed to better treat late-stage lenalidomide refractory patients

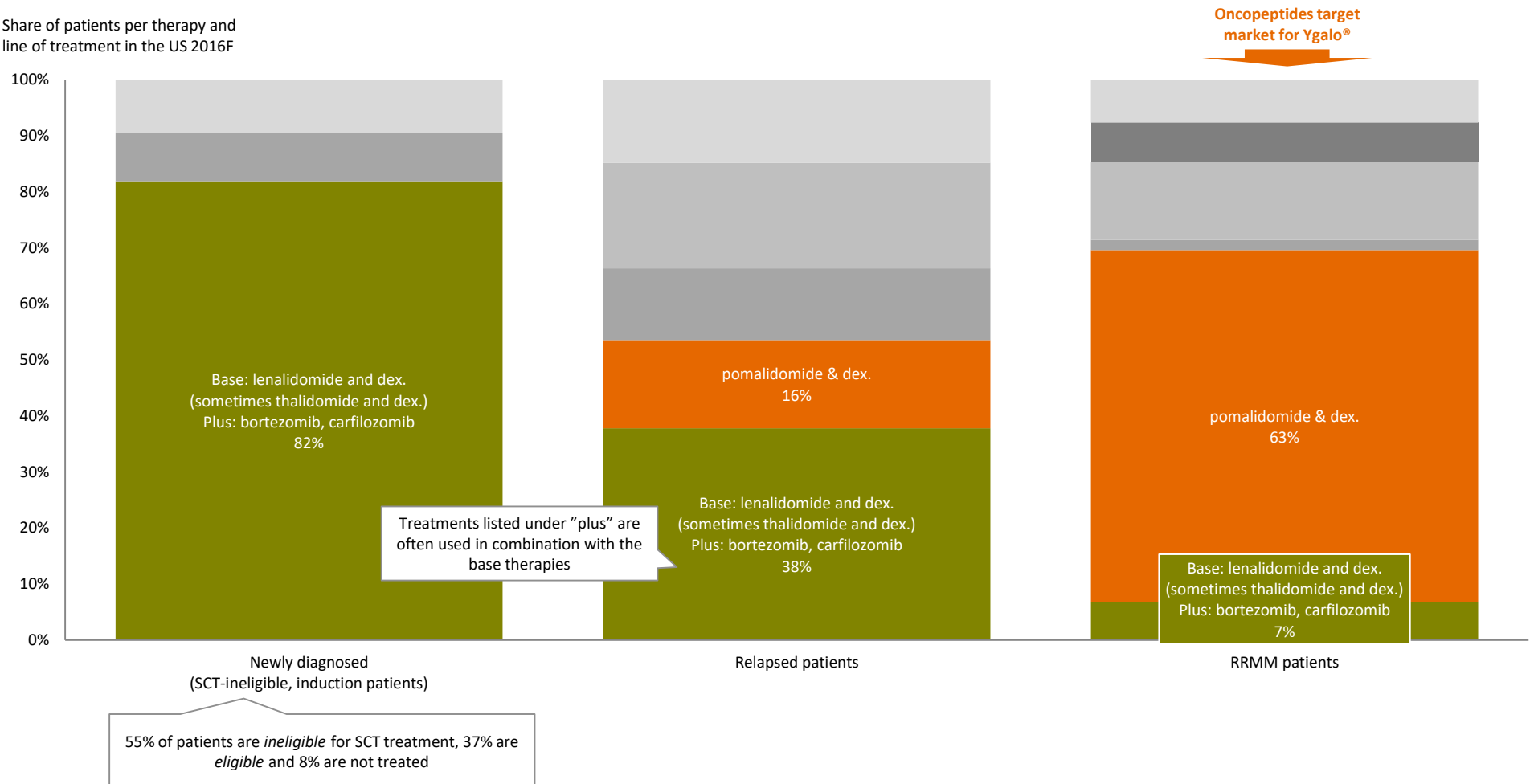
Source:

1) GlobalData. Sales figures could include sales for other diseases than multiple myeloma. Forecasts are based on equity analyst consensus collected by GlobalData

IMiDs are dominant across the different treatment settings – lenalidomide in newly diagnosed patients and pomalidomide in late-stage patients

Simplified overview of treatments used in different phases of multiple myeloma excluding stem cell transplantation

Share of patients per therapy and line of treatment in the US 2016F

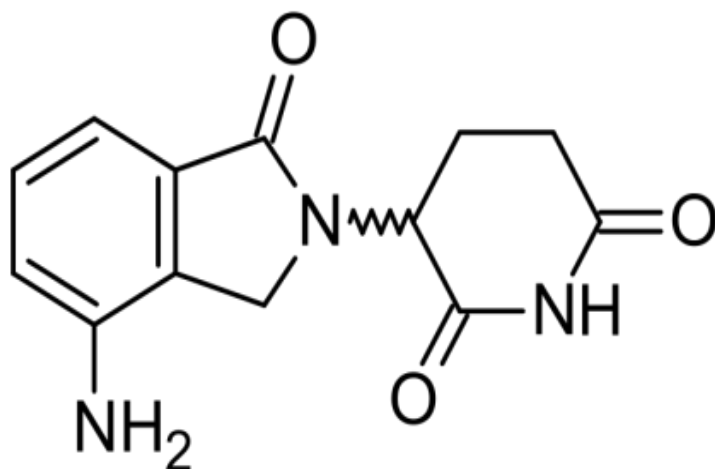


Source: GlobalData. Sources used by GlobalData in their estimation of patient shares per treatment: interviews with 10 KOLs and 90 prescribers across the 8 covered markets. Estimated shares of patients in the US 2016. Maintenance and consolidation treatment excluded (lenalidomide dominant).

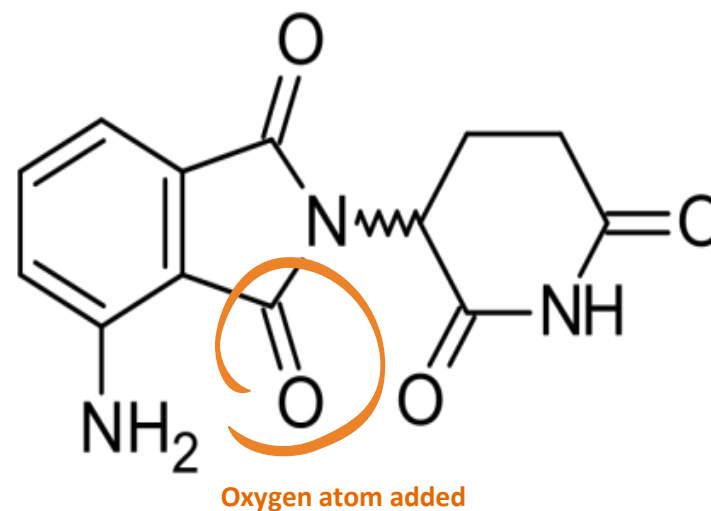
Lenalidomide and pomalidomide are sister molecules

Similar molecular structure from same library

LENALIDOMIDE



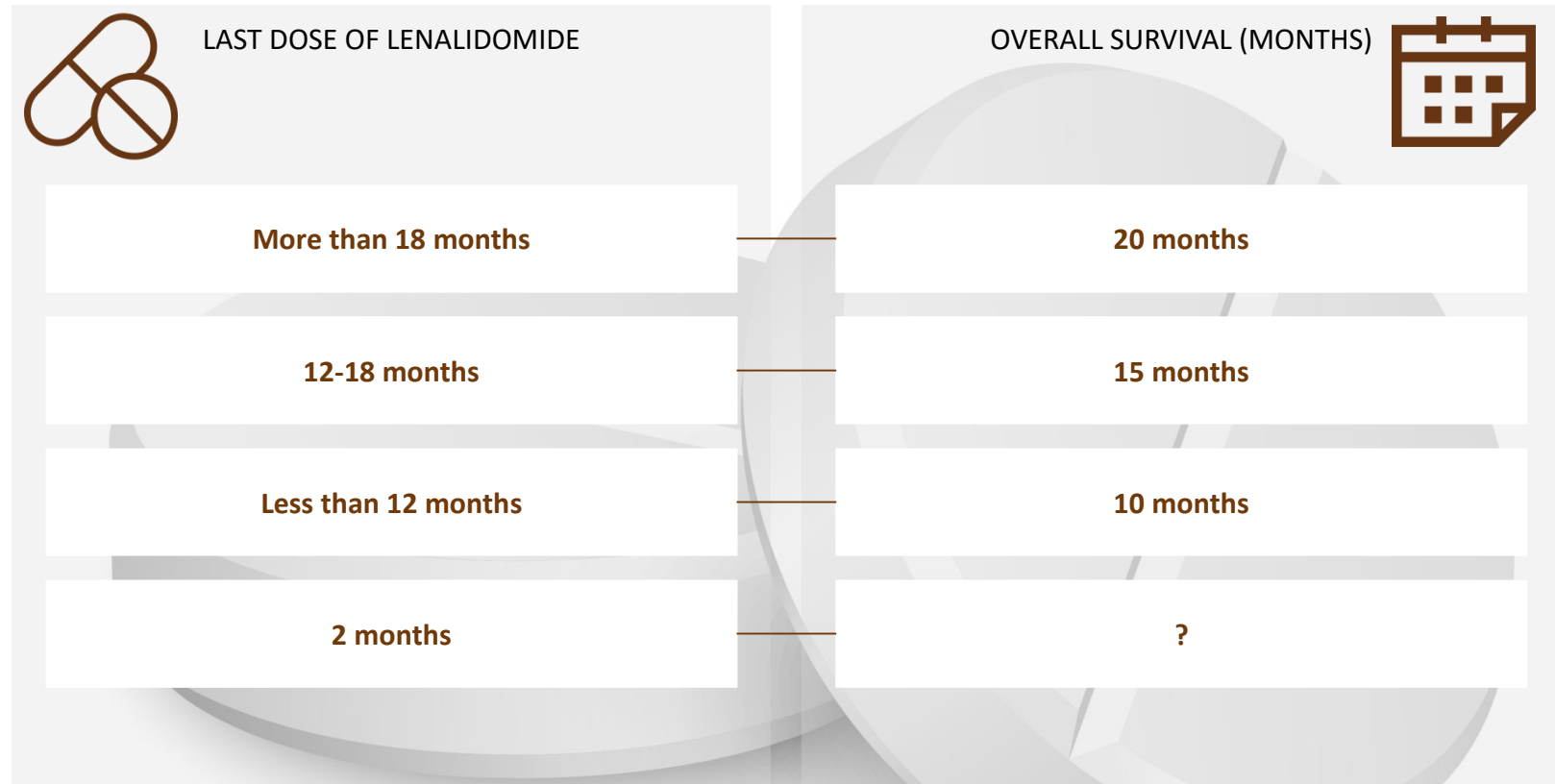
POMALIDOMIDE



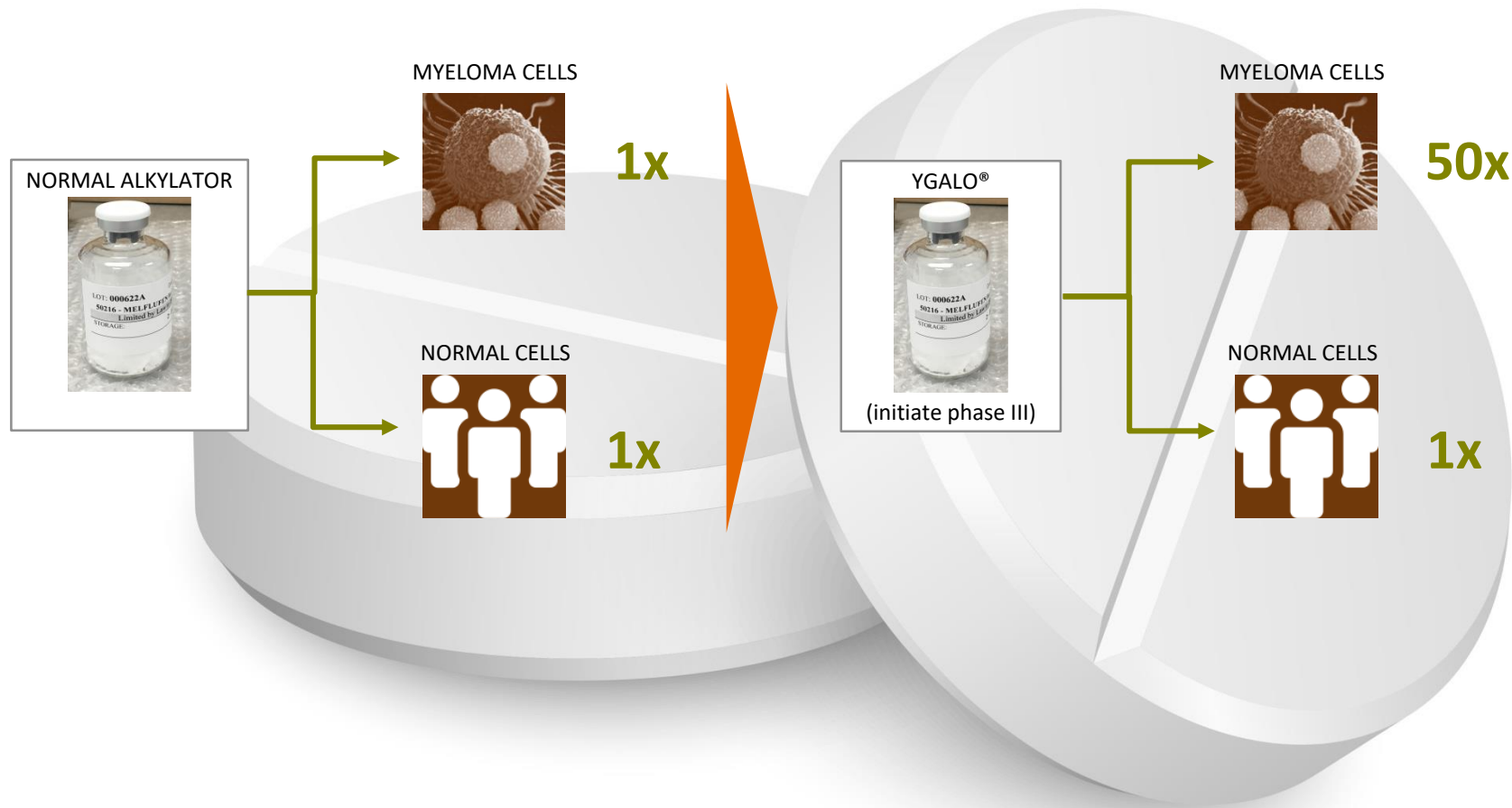
Cross-resistance between lenalidomide and pomalidomide up for discussion based on pre-clinical data as well as FDA and EMA scrutiny of investigator reported clinical data

Research presented at ASH 2016 argues for an IMiD free period

Dimopoulos research supporting an IMiD free period



Ygalo® – A novel peptidase potentiated alkylator for efficient and targeted treatment of haematological cancers



Current Phase II data supports superiority over standard of care in late-stage RRMM

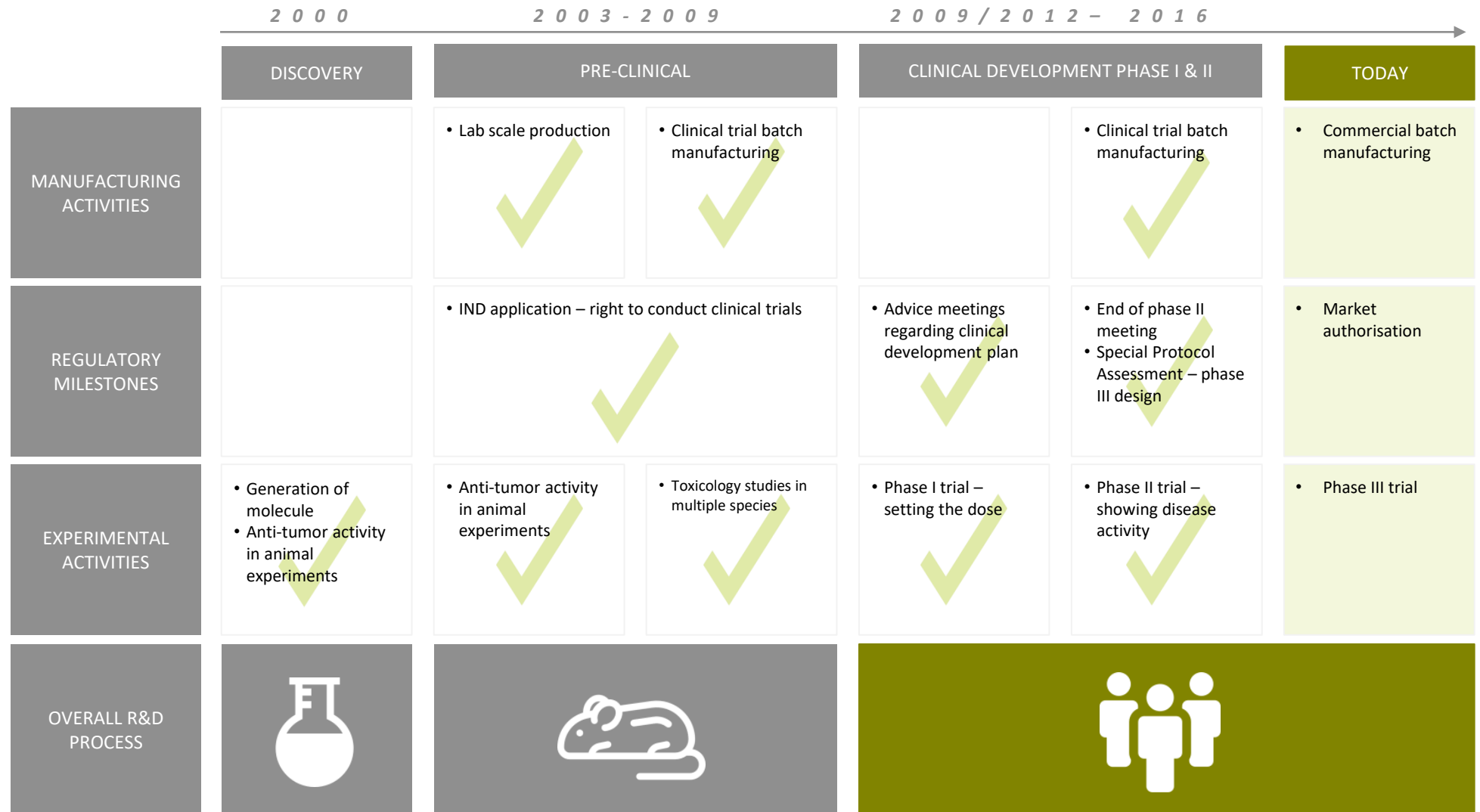
Comparison with data from patients that have not recently failed on lenalidomide

- 50% better Overall Survival
- 26% better Progression Free Survival (by hazard ratio)
- 28%-35% better objective tumour response rates (ORR and CBR)
- Better tolerated by the patients



Strong foundation for Phase III program design where Ygalo® will be directly compared to current standard of care

Ygalo® is a next generation targeted alkylator in the final stages of development



Key Opinion Leaders and regulatory interactions provides strong foundation for planned pivotal development program

KOL network consisting of leading oncologists within the field of MM

SELECTION OF ONCOPEPTIDES CLINICAL ADVISORS AND INVESTIGATORS



Prof. Paul Richardson – Dana-Farber Cancer Institute, Harvard, USA

- Clinical program leader and Director of Clinical Research at Jerome Lipper Multiple Myeloma Center (Dana-Farber Cancer Institute)
- Lead clinical investigator for bortezomib
- Lead clinical investigator for pomalidomide



Prof. Pieter Sonneveld – Erasmus University, Netherlands

- Professor and Head of Hematology at Erasmus University
- President-elect European Hematology Association
- Founder European Hematology Network
- Scientific advisory member for International Myeloma Foundation, International Myeloma Working Group and International myeloma Society

KEY OPINION LEADERS WORKSHOPS

Jan-12: Boston, US

Dec-13: New Orleans, US

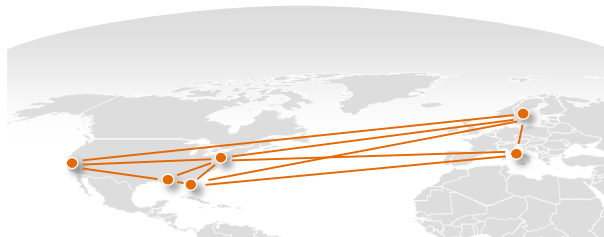
Jun-14: Stockholm, SE

Dec-14: San Francisco, US

Jan-15 to May-15: Individual Scientific Advice meetings with KOLs in EU and US

Sep-15: Rome, Italy

Dec-15: Orlando, US



Several regulatory interactions with meaningful authorities

FOOD AND DRUG ADMINISTRATION

Nov-12: Pre-IND type B meeting

Jan-13: IND application

Feb-13: IND approved

Mar-15: Orphan Drug Designation granted

Jun-15: Scientific Advice type C meeting

Dec-15: Scientific Advice type C meeting

Apr-16: Scientific Advice type C meeting

Jun-16: End of Phase II meeting

Jul-16: Application for exemption to conduct pediatric development under Pediatric Research Equity Act

Aug-16: Special Protocol Assessment Agreement Letter



NATIONAL AUTHORITIES (MHRA & SMPA)

May-04: Scientific Advice meeting with Swedish MPA

Feb-06: First phase I study application granted by Swedish MPA

Jan-13 to Dec-13: Permission granted to conduct clinical trials in DK, NL and IT

Apr-13: Phase I/II study application granted by Swedish MPA

May-14: Scientific Advice meeting with Swedish MPA

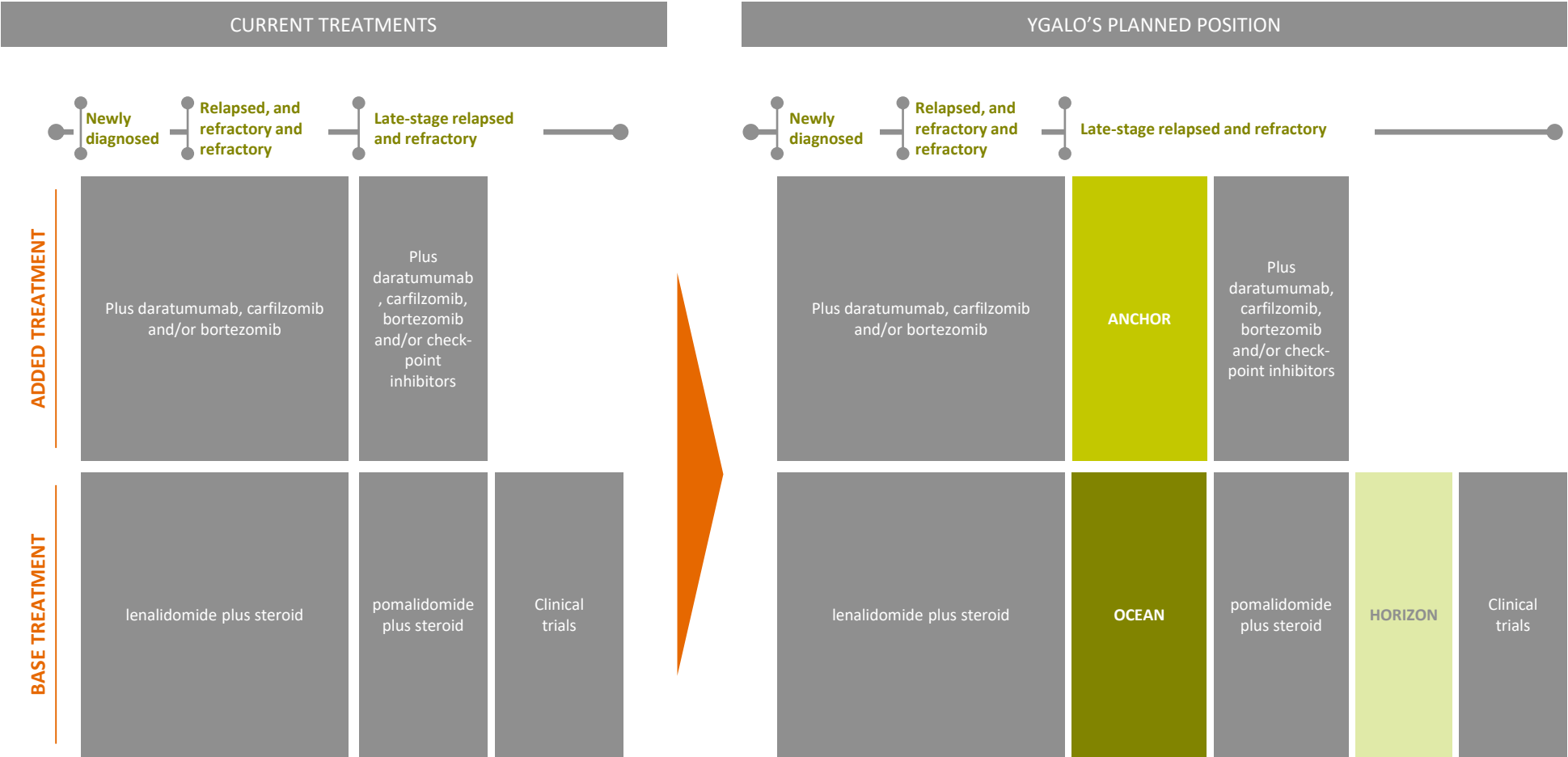
Mar-15: EU Orphan Drug Designation granted by COMP / EMA

Apr-15 to Nov-15: Several Scientific Advice meetings with Swedish MPA

Mar-16: MHRA (British Medicines and Healthcare Products Regulatory Agency) gives positive feedback on design of phase III study

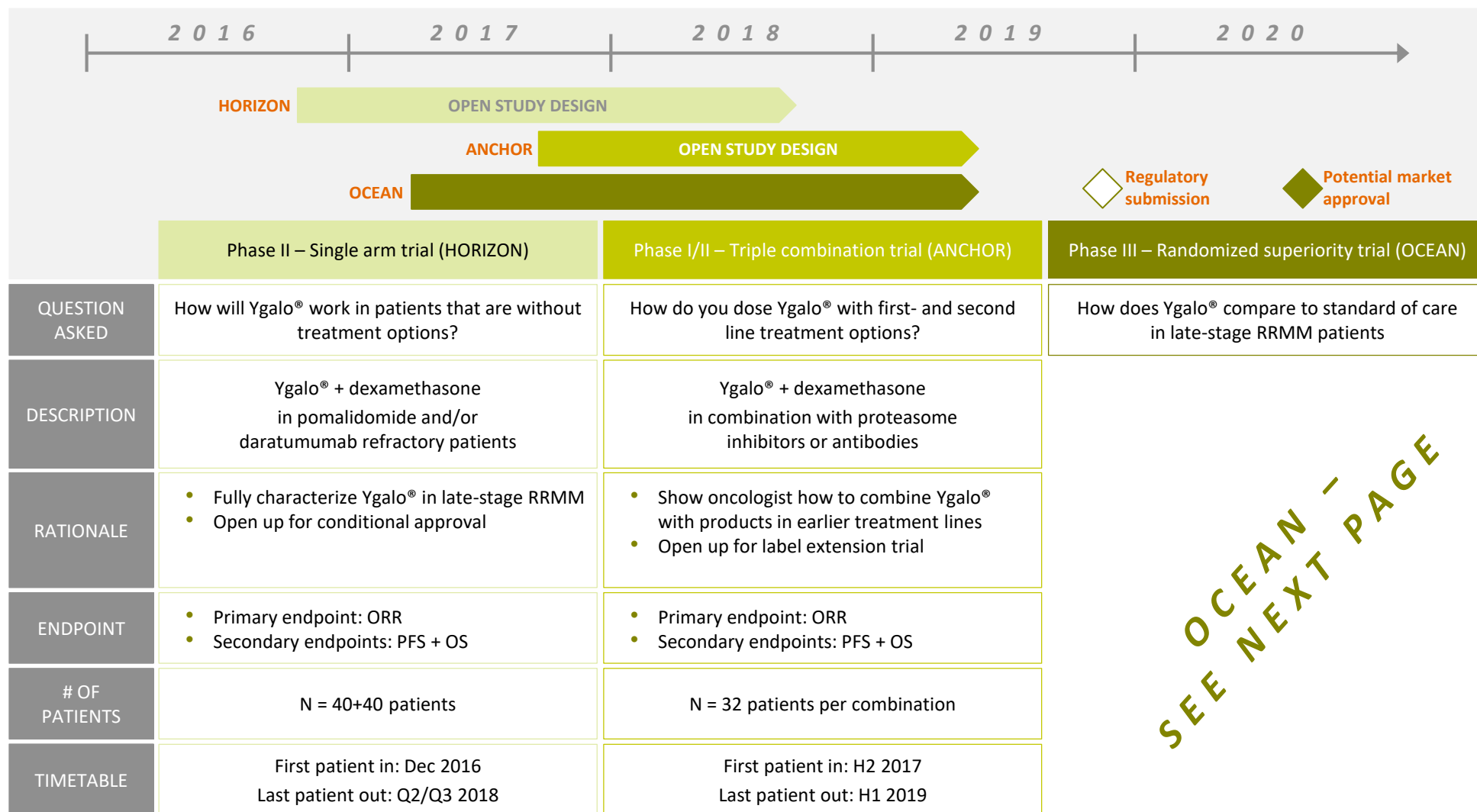


Clinical development program provides a complete data set to show how to use Ygalo® in late-stage RRMM



Full characterization of Ygalo® as a complement in late-stage RRMM will help increase physicians willingness to prescribe

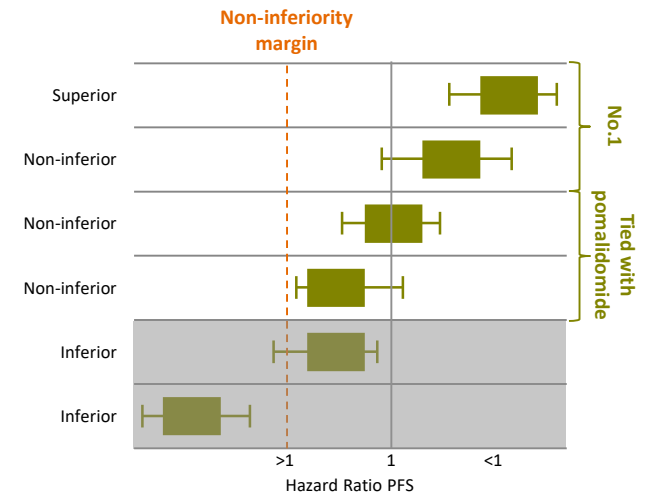
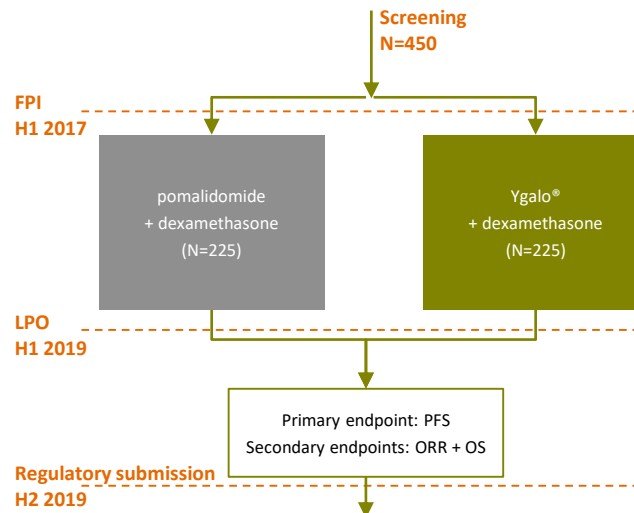
Regulatory approved and de-risked development program to characterize and maximize potential of Ygalo®



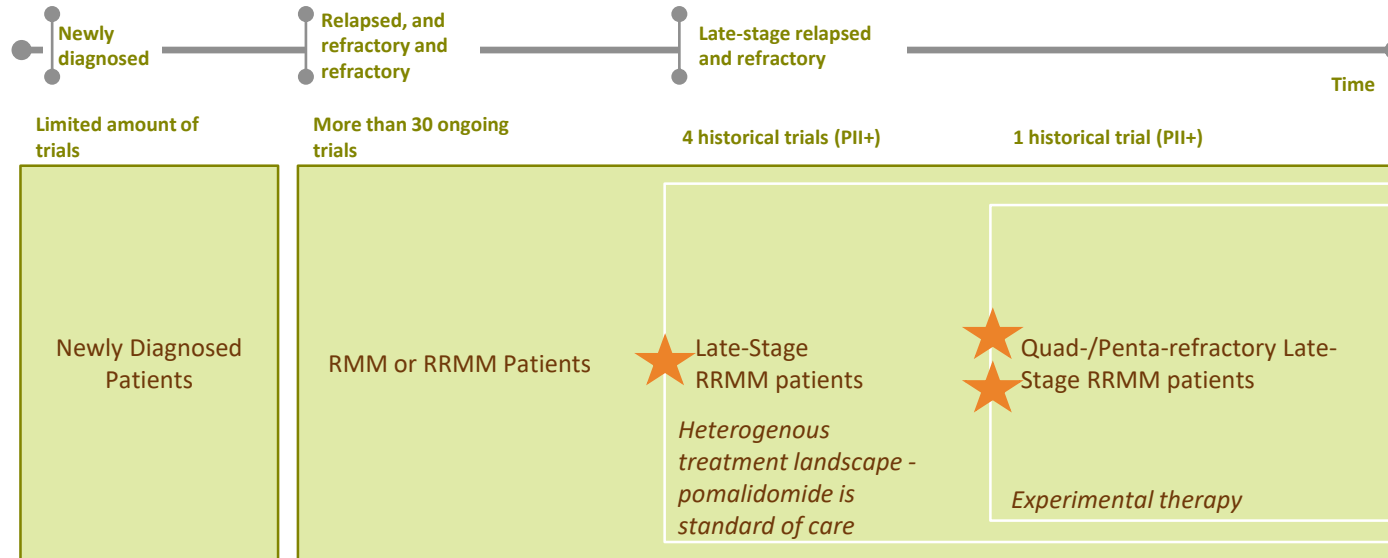
Pivotal phase III trial OCEAN will put Ygalo® head-to-head with pomalidomide in order to determine superiority

Despite aiming for superiority several aspects act to de-risk the phase III trial

BACKGROUND	OCEAN RATIONALE & DESIGN	POTENTIAL OUTCOMES
<ul style="list-style-type: none"> Lenalidomide used in first- and second-line treatment, pomalidomide used in third-line and late-stage relapse refractory patients Key de-risking argument: pomalidomide share – at least in part – resistance mechanism with lenalidomide Positive feedback and approval through FDA Special Protocol Assessment process Same lead clinical investigator in OCEAN as in Celgene's pivotal pomalidomide trial 	<ul style="list-style-type: none"> Randomized controlled trial comparing Ygalo® with pomalidomide in late-stage RRMM patients Purpose: To show superiority of Ygalo® over pomalidomide in late-stage RRMM Goal: Establish that Ygalo® is clinically superior to pomalidomide with statistical significance Inclusion criteria follows the pomalidomide label with one addition: Last line of therapy needs to have contained lenalidomide 	<ul style="list-style-type: none"> Superiority: Approval in US and EU Non-inferiority: Approval in EU



Myeloma – late-stage patients have limited options



Historical trial

results:

Ygalo (PII):

ORR: 23-29%

ORR: 30%

CBR: 34-37%

CBR: 50%

PFS: 3.6-3.7m

PFS: 4.3m

OS: 12-19m

OS: 18m



Historical trial

result:

ORR: 21%

CBR: 32%

PFS: 2.1m

OS: 9.3m



= 2+ prior lines of therapy, exposed to both PIs and lenalidomide, and disease progression while on therapy or within 60 days of last dose

Source:

1) Data derived from POMALYST FDA label (reference ID: 3953274)

2) Data derived from KYPROLIS FDA label (reference ID: 3161927)

3) Data derived from DARZALEX FDA label (reference ID: 3847807)

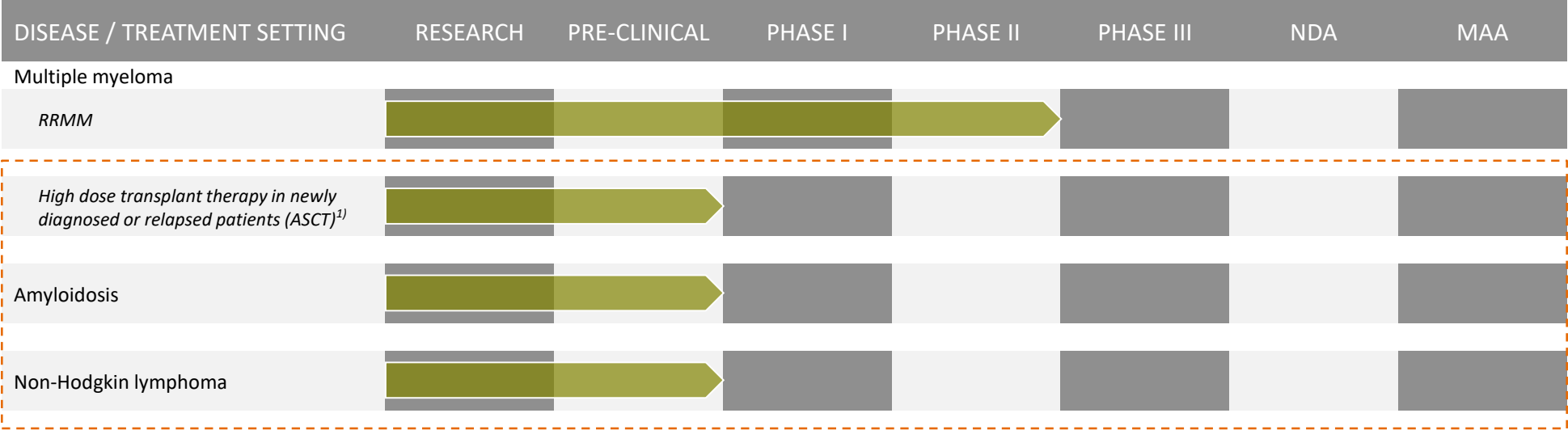
4) ASH 2016

5) EHA 2016



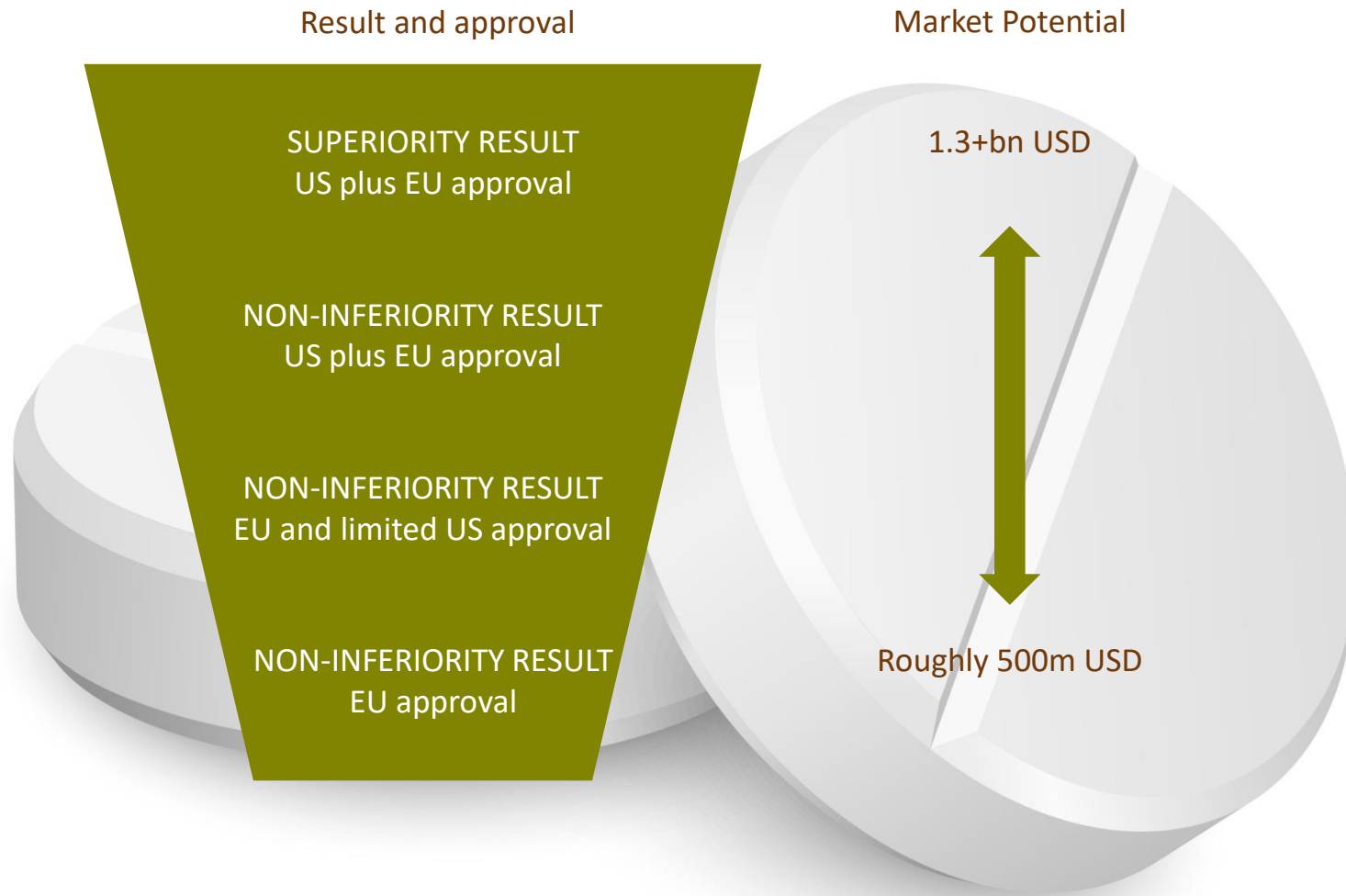
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Additional upside potential in high dose transplant therapy in MM, Amyloidosis and non-Hodgkin lymphoma



Source: Company information
Note:
1) Allows broadening of transplant indication outside of MM

Clinical development program design enables multiple paths to approval with different labels



Expected news flow until regulatory submission

CLINICAL DEVELOPMENT PROGRAM

- **Dec 2016:** First patient in HORIZON
- **During 2017:** Patient enrollment rate HORIZON
- **H1 2017:** First patient in OCEAN
- **H2 2017:** First patient in ANCHOR
- **H2 2017:** Patient enrollment rate OCEAN and ANCHOR
- **During 2018:** Patient enrollment rate OCEAN and ANCHOR
- **Q2/Q3 2018:** Last patient out HORIZON
- **H1 2019:** Last patient out OCEAN
- **H1 2019:** Last patient out ANCHOR

COMPANY RELATED

- **During 2018:** Presentation of commercialization strategy

CONFERENCES WHERE DATA COULD BE PRESENTED

- **Dec 2017:** American Society of Hematology (ASH)
- **Jun 2018:** European Hematology Association (EHA)
- **Jun 2018:** American Society of Clinical Oncology (ASCO)
- **Dec 2018:** American Society of Hematology (ASH)
- **Jun 2019:** American Society of Clinical Oncology (ASCO)
- **Jun 2019:** European Hematology Association (EHA)



EUROPEAN
HEMATOLOGY
ASSOCIATION



Highly experienced and dedicated management...

JAKOB LINDBERG
CEO



- CEO since 2011
- Med Lic in Molecular Immunology and MSc in pre-clinical medicine from the Karolinska Institute; BA in Finance and Administration
- Venture Partner at Patricia Industries in the Investor AB group
- Previous positions include: analyst for Merrill Lynch and consultant for McKinsey & Co; co-founder and CEO of Collectricon

BIRGITTA STÅHL
CFO



- CFO since 2016
- MSc Pharm from Uppsala University and MBA from University of Westminster
- Previous positions include: COO and acting CFO at Akinion Pharmaceuticals AB and KDev Oncology AB and VP Company Operations at Axelar AB

EVA NORDSTRÖM
VP Head of Clinical Development



- Head of Clinical since 2012
- MSc Pharm from Uppsala University and an Executive MBA from Stockholm School of Economics
- Previous positions include: Global Product Director and VP roles at Pharmacia and AstraZeneca based in Sweden and the USA

JOHAN HARMENBERG
CMO



- CMO since 2012
- Associate Professor, PhD and MD from the Karolinska Institute
- Previous positions include: CEO for Axelar and Akinion, CMO for Algeta, VP Development for Medivir and Global Medical Director for Pharmacia Upjohn
- Author of over 100 publications in scientific literature

ELISABETH AUGUSTSSON
Head of Regulatory Affairs



- Head of Regulatory affairs since 2015
- President and founder of Restracom
- MSc Pharm from Uppsala University
- Previous positions at Pharmacia&Upjohn, Medivir, Biovitrum, Karo Bio and Alexion

FREDRIK LEHMANN
Head of CMC



- Head of CMC since 2010
- PhD in Medicinal chemistry from Gothenburg University
- General Manager at Recipharm OT Chemistry AB
- Previously positions at: Pharmacia, Personal Chemistry, Biovitrum; has worked as an independent CMC consultant and co-founded six life science companies including Recipharm OT Chemistry AB

PAULA BOULTBEE
CCO



- CCO since October 2016
- Principal at PTB Consulting, Board president at the Max Foundation, Senior advisor to Monocl EGO AB,
- Registered nurse, B.A. in Health Science, Mälardalens Högskola, Clinical trial design and management, Lund University
- Previous positions include EVP of Sales & marketing at Pharmacyclis, EVP of Global marketing at Amgen

REIN PIIR
Head of Investor Relations



- Head of IR since October 2016
- CEO of Piir & Partners, Board member of Research Laboratories Sweden AB
- Business degree from Uppsala University
- Previous positions include : VP, Head of Investor Relations at Alligator Bioscience and Camurus. IR, CFO and EVP of corporate affairs at Medivir

...backed by strong board of directors and well renowned investors

ALAN HULME
Chairman



- Chairman of the Board since 2010
- Engaged across Europe since 2002 providing corporate development services on a consultancy basis to development stage client companies in the Life Sciences sector from his base in London
- Fellow of the Institute of Biomedical Sciences (UK)

JOHAN CHRISTENSON
MD, PhD
Board member



- Board member since 2012
- Partner at HealthCap
- Previous positions include: supervising the health care portfolio at SEB Företagsinvest, Odlander Fredrikson SA, Project director at AstraZeneca and Global Product Director and member of the global therapy area management team of Pain & Inflammation at Astra Zeneca
- Assistant Dean at the Karolinska Institute Graduate School for two years

JARL ULF JUNGNELIUS
MD, PhD
Board member



- Board member since 2011
- Advisor to Celgene and a board member of the Mesothelioma Applied Research Foundation Inc
- Previous positions include: Vice President of Oncology CR&D Solid Tumor Development at Celgene, Vice President of Oncology Clinical Development at Takeda, Pfizer and Eli Lilly & Company
- 25 years clinical and research experience from large pharmaceutical companies and academic organisations

JONAS BRAMBECK
PhD
Board member



- Board member since 2008
- Investment Manager at Industrifonden and a member of the board of directors in Oxthera AB, Cardoz AB and Nuevolution AS
- Previously positions at: AstraZeneca, Bruker Instruments, Nobel and others

PER SAMUELSSON
Board member



- Board member since 2012
- Partner at HealthCap
- Previous positions include: over 15 years investment banking experience, mainly with Aros Securities in Sweden which included, as Director in the corporate finance department where he specialized in Merger Transactions, Initial Public Offerings and Equity Incentive Programs

OLOF TYDÉN
Associate Prof., MD, PhD
Board member



- Board member since 2014
- Partner at Eureda, which he founded in 2000
- Previously positions include: Programme Director at the Medical Products Agency in Sweden, Medical Director at Leo Pharmaceuticals and Kabi-Vitrum and Senior Regulatory Adviser at Hoffman-La Roche.
- Previously expert to the European Commission in Health Telematics and Board member of Bioxell SpA, Aprea AB, Cantargia AB and Ximmune AB

LUIGI COSTA
Board member



- Board member since 2016
- CEO of Nordic Nanovector ASA
- Over 20 years of experience in international pharmaceutical and biotech industry
- Previous positions include: Vice President of EMEA for Onyx Pharmaceuticals where he led the company's international organization and the prelaunch and launch of Kyprolis® outside the USA, several leadership positions with Amgen (including Head of International Oncology Franchise)

CECILIA DAUN WENNBORG
Board member



- Board member since 2017
- Member of the board of directors in Getinge AB, Bravida Holding AB, ICA Gruppen AB, Loomis AB, Atvexa AB, Insamlingsstiftelsen Oxfam Sverige, Sophiahemmet AB and the non-profit organisation Sophiahemmet, Hotel Diplomat AB and CDW Konsult AB
- More than 14 years of experience as a member of the board and management in a number of sectors, e.g. the bank and care sectors, and has inter alia held positions as CEO of Skandia Link and Carema Vård & Omsorg AB and as deputy CEO of Ambea AB

Summary and concluding remarks

INVESTMENT HIGHLIGHTS

- 1 Ygalo® – A novel peptidase potentiated alkylator for efficient and targeted treatment of hematologic cancers, such as relapsed and refractory multiple myeloma (RRMM)
- 2 Well-defined orphan multi-billion USD market for late-stage RRMM characterized by limited treatment options and terminal outcome
- 3 Available phase II data supports superior efficacy and tolerability profile over standard of care in late-stage RRMM
- 4 Regulatory approved and de-risked development program to characterize and maximize potential of Ygalo®
- 5 Solid intellectual property position and Orphan Drug Designation from both FDA and EMA
- 6 Highly experienced and dedicated management backed by well-renowned investors
- 7 Additional upside potential in high dose transplant therapy in MM, Amyloidosis and non-Hodgkin lymphoma

A microscopic image showing a cluster of cells, with one cell in the foreground being more detailed and textured than the others in the background.

Thank you for your time