

Nomenclature

International non-proprietary name (INN)

Melphalan flufenamide

Chemical name

4-[Bis-(2-chloroethyl)amino]-L-Phenylalanine-4-fluoro-L-phenylalanine ethyl ester hydrochloride

Laboratory codes

Melflufen hydrochloride

J1

CK 1535

CAS No.

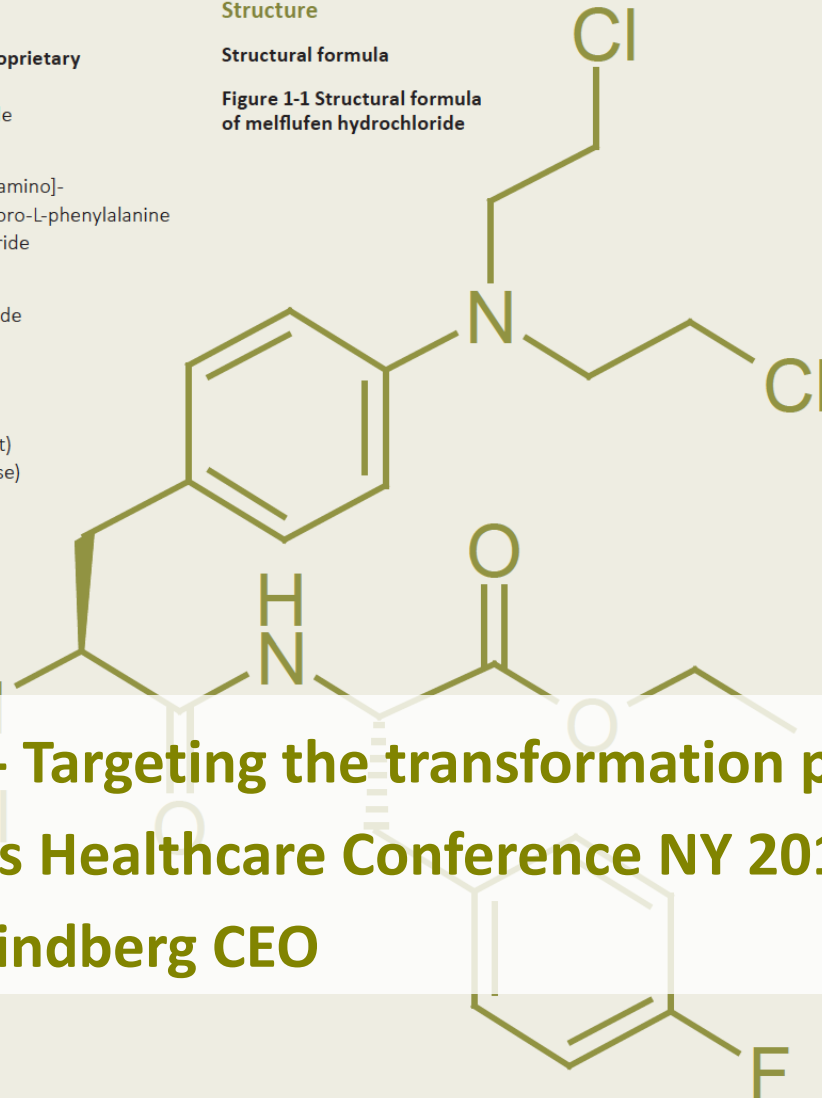
380449-54-7 (HCl salt)

380449-51-4 (free base)

Structure

Structural formula

Figure 1-1 Structural formula of melflufen hydrochloride



Molecular formula

C₂₄H₃₁Cl₃N₃O₃ (HCl salt)

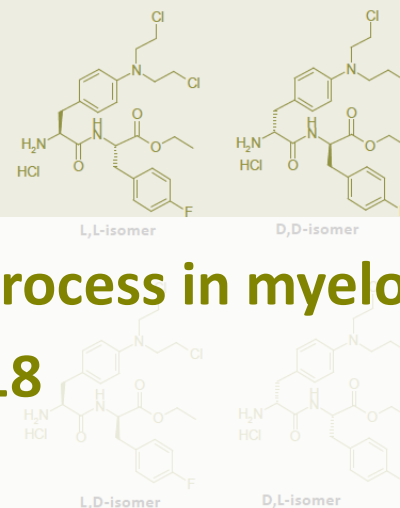
Molecular weight

534.9 (HCl Salt)

Stereochemistry

Melflufen hydrochloride contains two stereogenic centers giving rise to four possible stereoisomers. Melflufen hydrochloride drug substance is the L,L-isomer. The structures are outlined in Figure 1-2.

Figure 1-2 Structure of melflufen hydrochloride isomer



General properties

Appearance

White to slightly yellowish powder

Solubility

Melflufen hydrochloride is soluble in most organic solvents. The solubility in water and buffers is limited.

Partition coefficient

ClogP = 4.04 (tecken) 0.66, calculated using ACD logP DB, v.6.0 (from Advanced Chemistry Development)

Dissociation constant

pKa 10.0 (determined in ethanol solution)

Optical rotation

[α]_D 5.2° (c 1.9, CH₃OH) at 20°C

Thermal behaviour

Differential scanning calorimetry (DSC) was performed using a Mettler Toledo DSC 822 instrument and a scanning rate of 2(tecken)C/minute. The melting temperature was measured using batch GF404528 and determined from the DSC thermogram to be 205.4°C, as shown in Figure 1-3.

Ygalo[®] - Targeting the transformation process in myeloma

Jefferies Healthcare Conference NY 2018

Jakob Lindberg CEO

Disclaimer

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Oncopeptides overview

Ongoing phase 3 program addressing a USD 2bn+ market in myeloma

- Located in Sweden (Stockholm) and US (Bay Area)
- IPO in February 2017 on Nasdaq OMX in Stockholm
- Market cap: approximately 660 MUSD
- Cash position end of Q1 2018: 76 MUSD
- Burn rate Q1 2018: 8 MUSD
- Experienced management team
- Most advanced indication is myeloma. Programs currently running more than 100 hospitals in the US, EU and Israel

- Phase 3 read-out Q3 2019
- New indications and NCEs in development with clinical trials possibly starting in 2019



Ygalo[®] is targeting the transformation process in myeloma

Phase III read-out Q3 2019

Ygalo[®]: a peptidase enhanced compound for late stage RRMM

- Ygalo[®] is an alkylating peptide belonging to the Peptidase Enhanced Class (PEnC) of compounds
- The PEnC motif gives rise to selective cytotoxicity through targeting of transformed cells
- Phase 2 clinical data supports superior efficacy and tolerability over current standard of care in late-stage myeloma
- Do not share resistance mechanisms that impact current therapies (IMiDs, PIs, alkylators and anti-CD38)

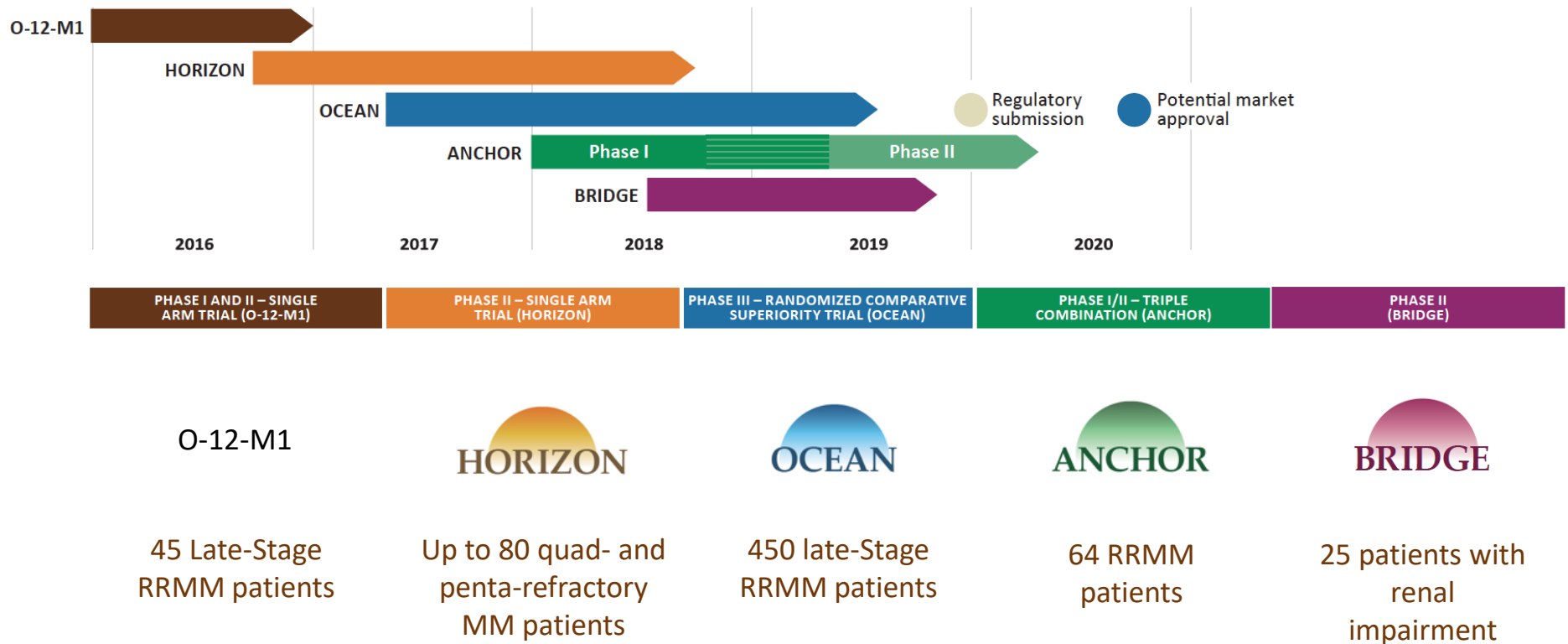
Significant unmet need and growing patient population

- Relapse is inevitable. New targeted therapies grow the market opportunity by increasing disease control in earlier lines of therapy
- Prognosis is poor, with limited options available in late-stage disease
- Ygalo[®] is addressing a \$2bn+ ¹⁾ late-stage disease market with double digit % growth

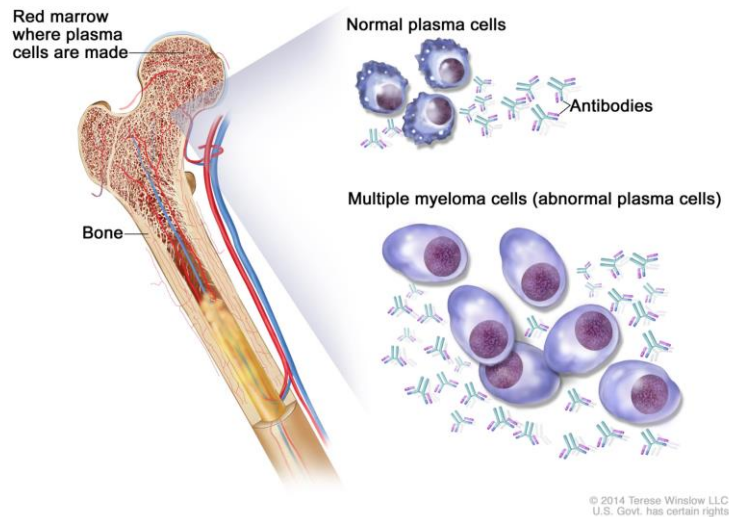
Fully funded pivotal Phase 3 trial underway; broad development program

- Agreement with FDA (SPA) and EMA on P3 clinical trial design
- Orphan drug designation in EU and US
- Multiple paths to approval de-risk the development pathway
- Good activity signal in a broad range of oncology indications

Overview of clinical development program in late-stage multiple myeloma



Multiple Myeloma is a hematological cancer with no cure



- Median OS just over 5 years and increasing
- Clonal selection results in inevitable relapse and resistance development
- New agents are growing the patient population in later lines of therapy (number of 4th+ line patients receiving treatment in the US grew by more than 40% in 2017)
- Later line patients have very few treatment options – pomalidomide is the de facto standard-of-care once a patient starts to respond poorly to lenalidomide, PIs and anti-CD38 based therapies (USD1.6bn in pomalidomide sales 2017 with 20%+ growth)
- Lack of ubiquitously expressed antigens in myeloma means that antibody based therapies are used together with IMiDs, PIs and alkylators outside of a rescue treatment setting (to ensure that all clones get some level of treatment)

Myeloma is primarily treated with single agents¹⁾; lenalidomide and bortezomib are dominant (USA 2017)

Pomalidomide is the primary choice after lenalidomide and proteasome-inhibition failure

Number of patients treated per 12m²⁾

Lenalidomide	55,565
Bortezomib	52,289
Daratumumab	17,068
Carfilzomib	15,133
Pomalidomide	13,459
Ixazomib	10,843
Other	22,305
<hr/>	
Total # of patients treated	132,829

- 9 out of 10 patients receive broad spectrum agents (IMiDs, PIs and/or alkylators)
- Agents with problematic tolerability/safety profile and/or complicated administration schedule get limited traction relative to efficacy profile (e.g. panobinostat with GI toxicity and carfilzomib with cardio-toxicity as well as complex administration schedule)
- Majority of patients receive only single agent treatment (combination treatment is only dominant in the 1st line setting)

35% of US 1st line patients received single agent treatment 2017

60% of US 2nd line patients received single agent treatment 2017

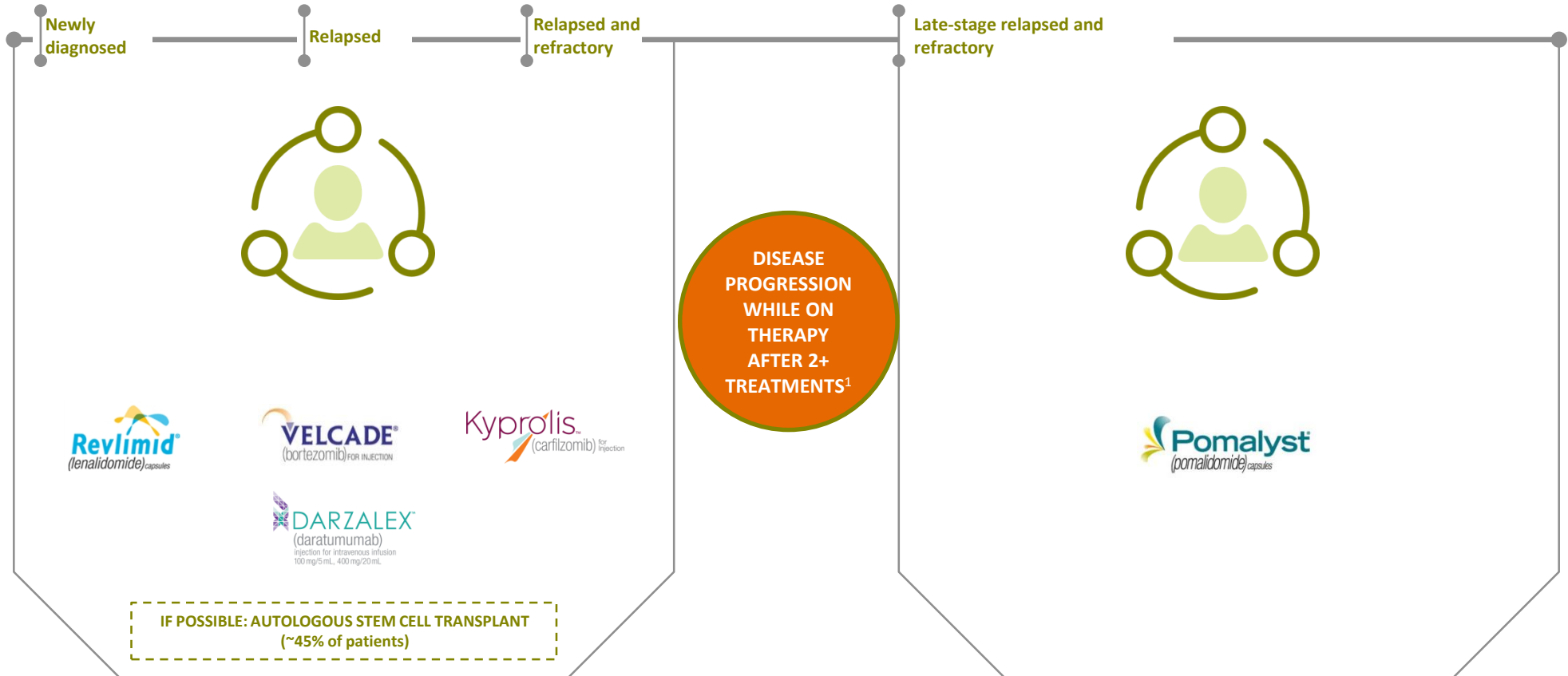
- Lenalidomide and proteasome inhibitors (PIs) are used early in the treatment algorithm. Daratumumab is moving from last-line to 1st line/ 2nd line rapidly
- Pomalidomide is the primary choice after lenalidomide and PI failure (disease progression while on therapy)

1) Not counting steroids

2) IntrinsicQ and Kantar Health.

Late-stage myeloma patients are well defined from both a regulatory and clinical point of view

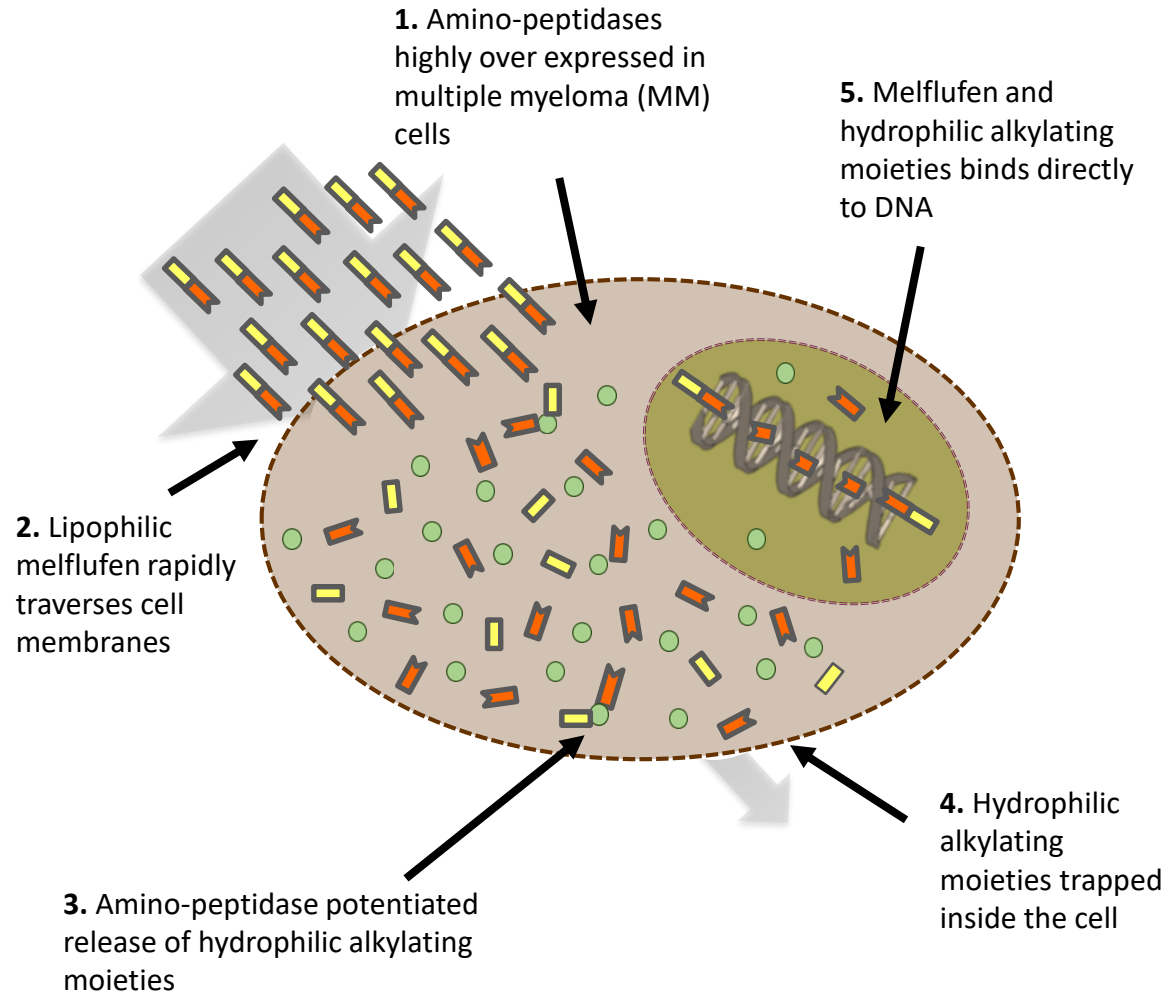
Lines of therapy throughout the disease stages¹⁾



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

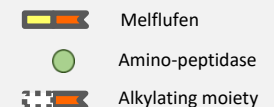
Ygalo[®] is an alkylating peptide targeting transformed cells

Amino-peptidases are overexpressed up to 250x as part of transformation process –
CD13 and matrix metallopeptidases are the most well characterized members of family



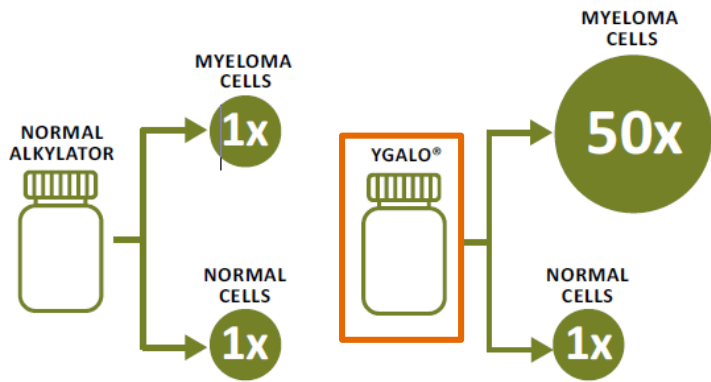
Peptidase enhanced activity in MM cells results in:

- Approx. 50-fold higher intra-cellular exposure in MM cells^{1,5}
- Approx. 50-fold higher anti-MM potency^{1,2,5}
- Alkylation of DNA with limited or no induction of DNA repair^{3,5}
- Strong anti-angiogenic properties^{1,4,5}
- Increase in therapeutic index of 20x – 40x (MM cells compared with peripheral blood mononuclear cells)^{1,5}



Selective cytotoxicity has translated well into the clinic

Phase II data supports superiority of Ygalo® over standard-of-care in late-stage myeloma - a \$2bn+ market opportunity



- >75% better Overall Survival (best survival data to date in late-stage myeloma)
- 30% better Progression Free Survival (by Hazard Ratio)
- 25%-35% better objective tumour Response Rates (ORR and CBR)
- Better tolerated by the patients – non-haematological toxicity is rare

Strong foundation for Phase III program design where Ygalo® is directly compared to pomalidomide

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

Late-Stage Relapsed Refractory



TREATMENT	ORR	CBR	MEDIAN PFS	MEDIAN DOR	MEDIAN OS
Pomalidomide + dexamethasone	24%	NR	3.6 months	7.0 months	12.4 months
Ygalo® + dexamethasone	31%	49%	5.7 months	8.8 months	20.7 months

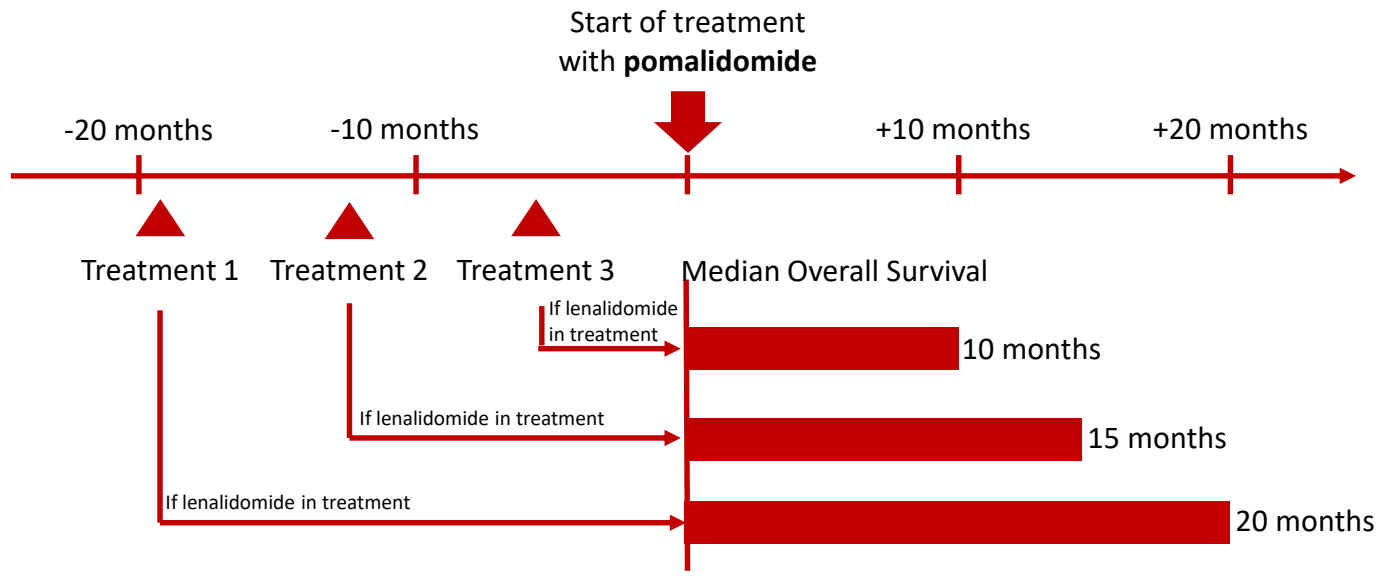
Note: NR=Not Reported. Ygalo® is not market approved.

Source: FDA Label.

However, the cross-resistance between pomalidomide and lenalidomide puts pomalidomide at a disadvantage in the real-world setting (see OCEAN trial design)

Pomalidomide shares resistance mechanism with lenalidomide – no assumption has been made in OCEAN power calculation about this factor

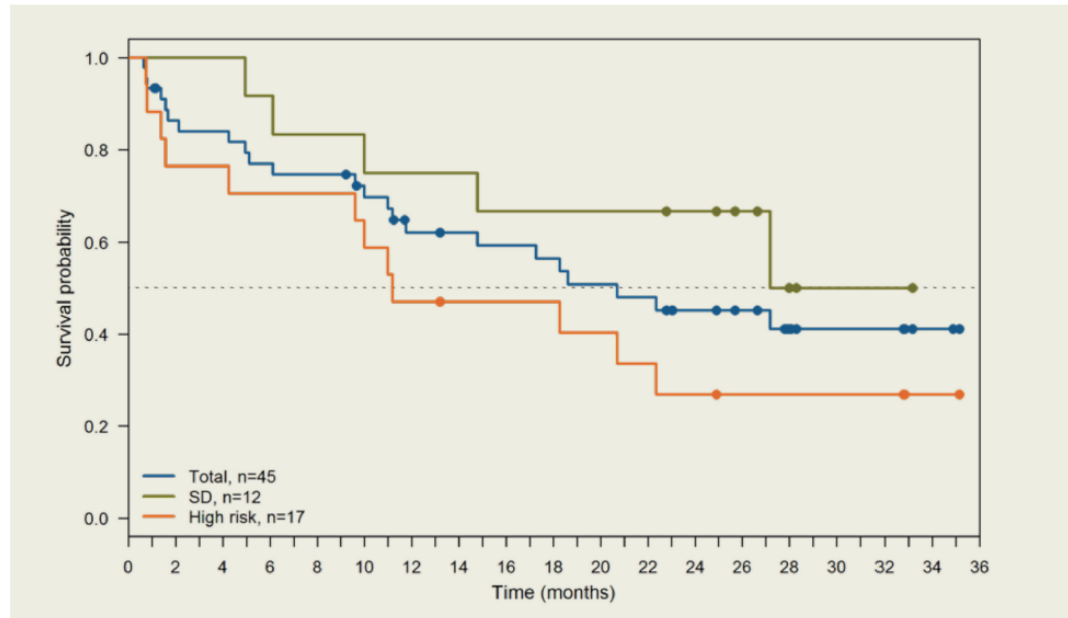
Dimopoulos research supporting an IMiD free period



50% reduction in efficacy if patient recently failed on lenalidomide - suggests significant resistance overlap between lenalidomide and pomalidomide

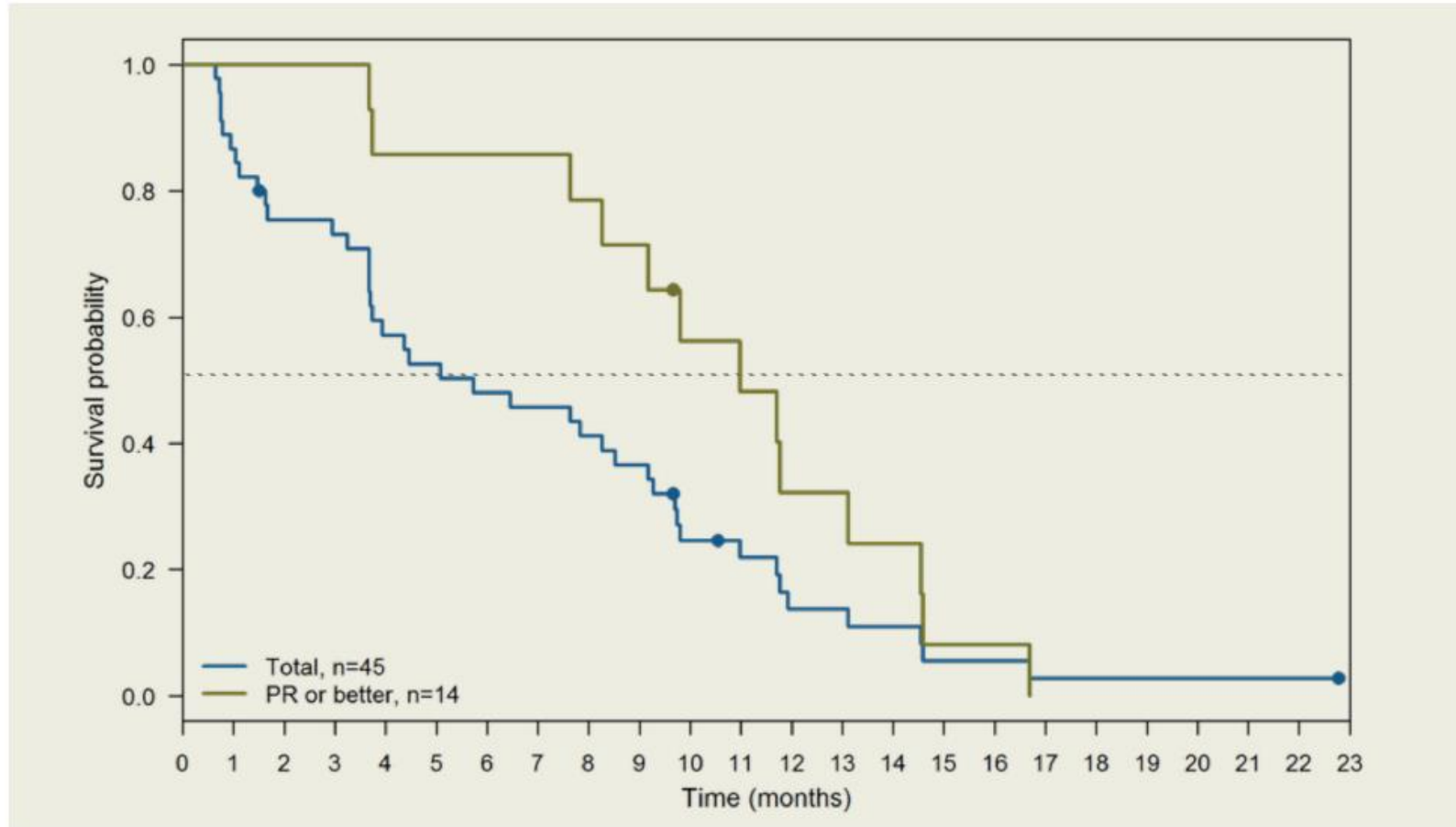
Best-in-class survival data from Ygalo[®] in our phase II study (O-12-M1) in late-stage RRMM

Figure 2. Overall survival



- Best-in-class survival data in late-stage RRMM
- Best PFS to date for a broad-spectrum agent in late-stage RRMM
- Tolerability profile very favorable with patients experiencing comparatively few side-effects that are detrimental to QoL (which in a palliative care setting with elderly patients is key)

Best Progression Free Survival data from any broad-spectrum agent in late-stage RRMM



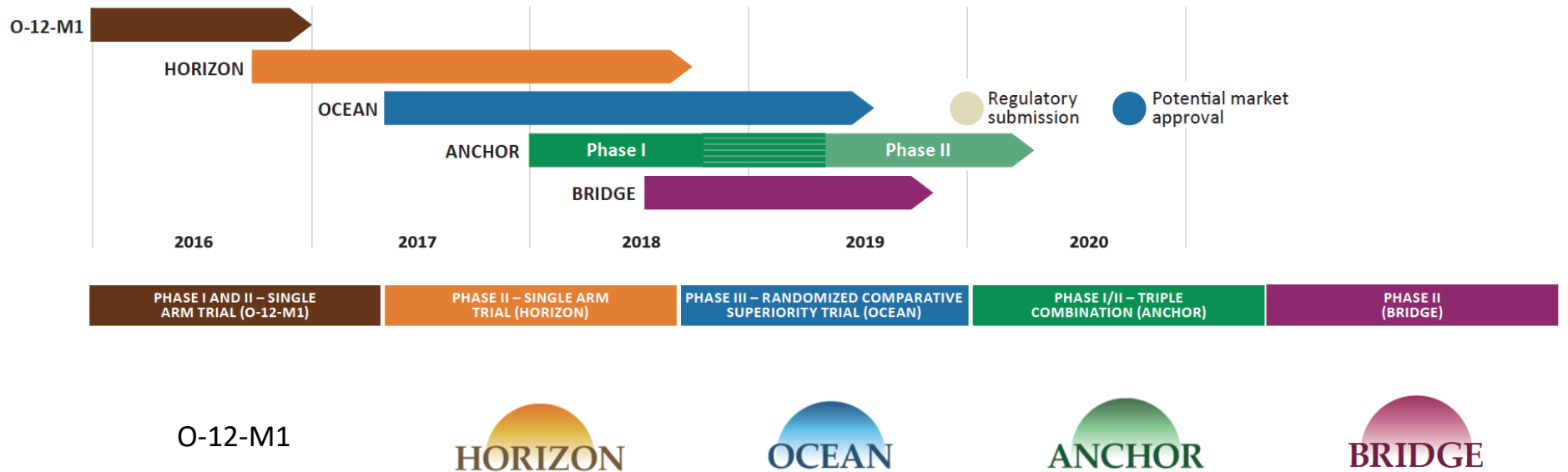
Excellent tolerability – key in a palliative care setting

Table 6. Treatment-related G3/4 AEs occurring in ≥ 5% of the patients (N=38)

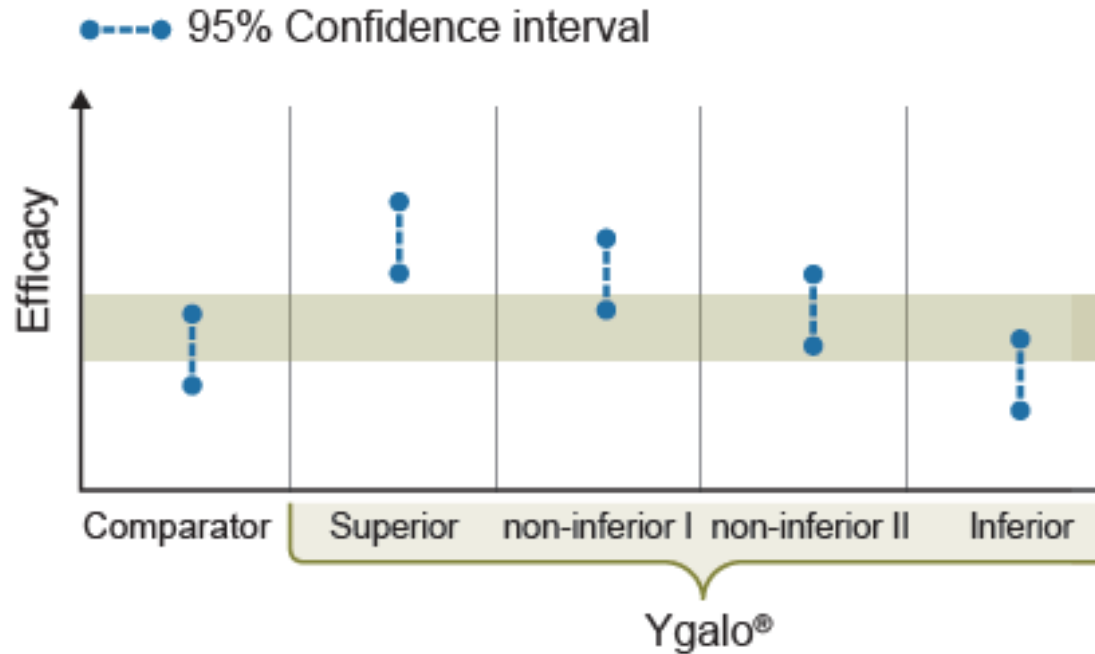
	GRADE 3 OR 4, n (%)	GRADE 4, n (%)
Any treatment-related AE	22 (58)	15 (39)
Blood and lymphatic system disorders	20 (53)	14 (37)
Thrombocytopenia	17 (45)	12 (32)
Neutropenia	15 (39)	9 (24)
Leukopenia	3 (8)	3 (8)
Anemia	8 (21)	0
Lymphopenia	3 (8)	0
Hemolytic anemia	2 (5)	0

- No treatment related gr3/4 AEs outside of the hematological compartment
- Good indication of Quality of Life while on treatment

Our clinical development program



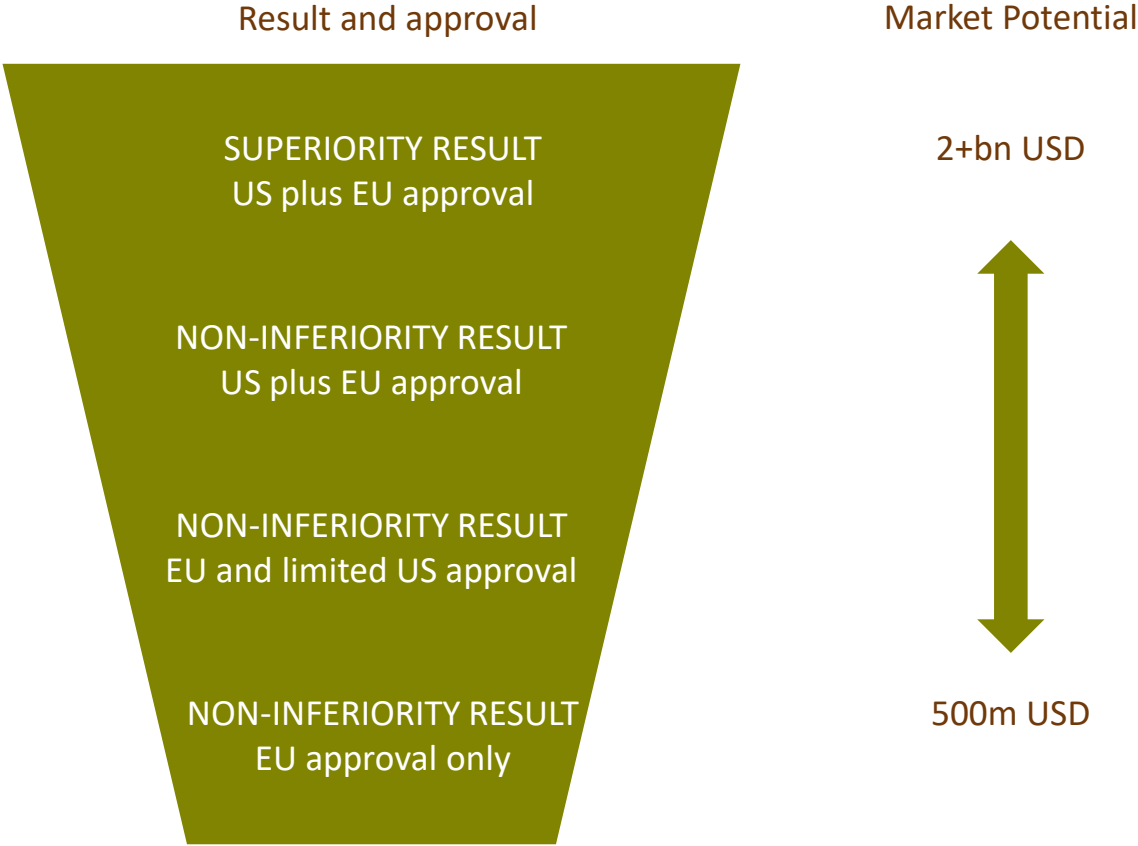
HORIZON and BRIDGE support the result in OCEAN



In a non-inferiority outcome scenario, differentiation is key, e.g.

- Better tolerability (OCEAN)
- No overlap in resistance mechanism (HORIZON)
- Renal clearance not required for Ygalo® (BRIDGE)

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



Evolution of our myeloma treatment position



2+ prior lines, IMiD and PI exposed, and progressive disease while on therapy or within 60 days of last dose in last line of therapy (OCEAN)



IMiD and PI exposed with poor response development (ANCHOR will guide us)



IMiD and PI exposed (ANCHOR will guide us)

Executive summary

- Ygalo as a 30 min once monthly infusion has showed best-in-class efficacy with excellent tolerability in late-stage RRMM patients
- Phase III study is 90% powered to show superiority of Ygalo in late-stage RRMM
- In OCEAN, all patients have recently failed on lenalidomide based therapy
- OCEAN, HORIZON and BRIDGE are designed to maximise the data-set with regard to differentiation vis-à-vis pomalidomide (especially in a non-inferiority outcome scenario)
- Top-line data from the P3 study OCEAN in Q3 2019
- New NCEs as well as indication broadening programs for Ygalo under development