

# BerGenBio ASA (OSE: BGBIO) Results Fourth Quarter & FY 2018

12<sup>th</sup> February 2018

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# Introduction & recent highlights



# BGBIO – Investment Highlights



## AXL inhibitors – potential cornerstone of cancer therapy

Leaders in developing selective AXL inhibitors

Bemcentinib (Ph2), AXL-antibody BGB149 (Ph1), AXL ADC ADCT-601 (Ph1)

AXL is a novel oncology target to overcome immune evasion, therapy resistance & spread

**Pipeline opportunities in multiple cancers and fibrosis**



## Ph2 data in AML & NSCLC with selective AXL inhibitor bemcentinib

Monotherapy and combinations with immune-, targeted and chemotherapies

Biomarker correlation across programme, parallel CDx development

AXL positive patients:

**43% ORR in R/R AML/MDS** (monotherapy)  
**40% ORR in 2L NSCLC** (KEYTRUDA combo)

**Late stage clinical trials to start H2'19**



## Resourced to deliver significant milestones

Clinical trial collaborations with Merck and leading academic centres

AXL antibody out licensed to ADC Therapeutics SA

38 staff at two locations:  
HQ & R&D in Bergen, Norway;  
Clinical Development in Oxford, UK

**Cash NOK360m**

# Agenda

1. Introduction and recent highlights
2. Bemcentinib: First-in-class highly selective AXL inhibitor in Phase II
3. Clinical Development Opportunities
4. BGB149 – Anti AXL monoclonal antibody in Phase I
5. Finance
6. Outlook

# Q4 2018 Highlights

## **Bemcentinib monotherapy & combo in AML/MDS (BGBC003):**

PoC monotherapy data reported at ASH, combination studies ongoing

## **Bemcentinib + KEYTRUDA® in NSCLC (BGBC008):**

Selected as late-breaking abstract for SITC – PoC phase II data (stage 1)

## **Bemcentinib biomarker programme:**

Selected for poster discussion at ESMO

## **Strengthened clinical development team**

## **Post-period updates:**

BGB149 (AXL antibody) and ADCT-601 (partnered AXL ADC): start phase I trial



# Increasing profile and recognition of bemcentinib at international clinical congresses in 2018

January	February	March	April	May	June	July	August	September	October	November	December	
<div>✓</div> <div><b>ASCO-SITC</b> Lung cancer, TNBC and AML trial update</div> <div><ul style="list-style-type: none"><li>✓ KEYTRUDA combo well tolerated</li><li>✓ Bemcentinib induces diversification of T-cell receptor repertoire (AML)</li></ul></div>	<div>✓</div> <div><b>AACR</b> Preclinical Update</div> <div>Bemcentinib increases efficacy of checkpoint inhibitors</div>	<div>✓</div> <div><b>ASCO</b> NSCLC, AML, Melanoma and biomarker update</div> <div>Bemcentinib enhances responses to<ul style="list-style-type: none"><li>✓ IO,</li><li>✓ chemo,</li><li>✓ targeted therapies</li><li>✓ and has monotherapy efficacy</li></ul></div>	<div>✓</div> <div><b>EHA</b> AML trial update</div> <div>Responses to bemcentinib monotherapy correlated with AXL biomarker</div>	<div>✓</div> <div><b>WCLC</b> Lung cancer trials update</div> <div><ul style="list-style-type: none"><li>✓ 40% ORR in AXL+ pts in combo w/ KEYTRUDA</li><li>✓ Improved PFS in combo with erlotinib and chemo</li></ul></div>	<div>✓</div> <div><b>ESMO</b> Biomarker update</div> <div><ul style="list-style-type: none"><li>✓ AXL biomarkers identified</li><li>✓ Melanoma clinical update</li><li>✓ AXL's role in low-risk MDS (pre-clinical)</li></ul></div>						<div>✓</div> <div><b>SITC</b> NSCLC data late breaking</div> <div><b>Late-breaking abstract:</b> 5.9m PFS in AXL+ previously treated NSCL in combo w/ KEYTRUDA (c80% improvement in AXL+ pts vs AXL-)</div>	<div>✓</div> <div><b>ASH</b> AML trial data update</div> <div>43% CR/Cri/CRp rate in AXL biomarker positive pts</div>



**Key data presented in Q4 supports future strategy for late-stage clinical development of bemcentinib in AML/MDS and NSCLC**

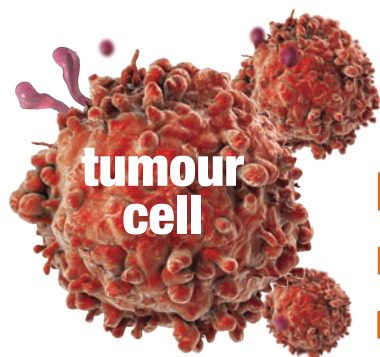


# AXL drives aggressive cancer





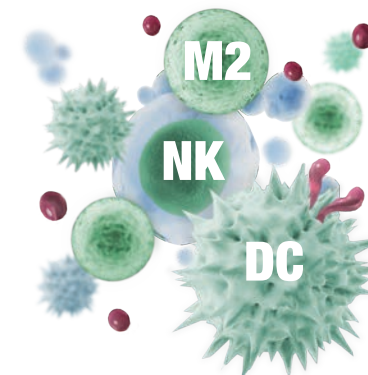
# AXL receptor tyrosine kinase drives aggressive disease including therapy resistant, immune-evasive tumours



**Drives tumour cell plasticity:  
non-genetic resistance  
mechanism**

AXL drives features of aggressive cancer:

- Acquired therapy resistance
- Immune escape
- Metastasis



**Key suppressor of innate  
immune response**

AXL is an innate immune checkpoint:

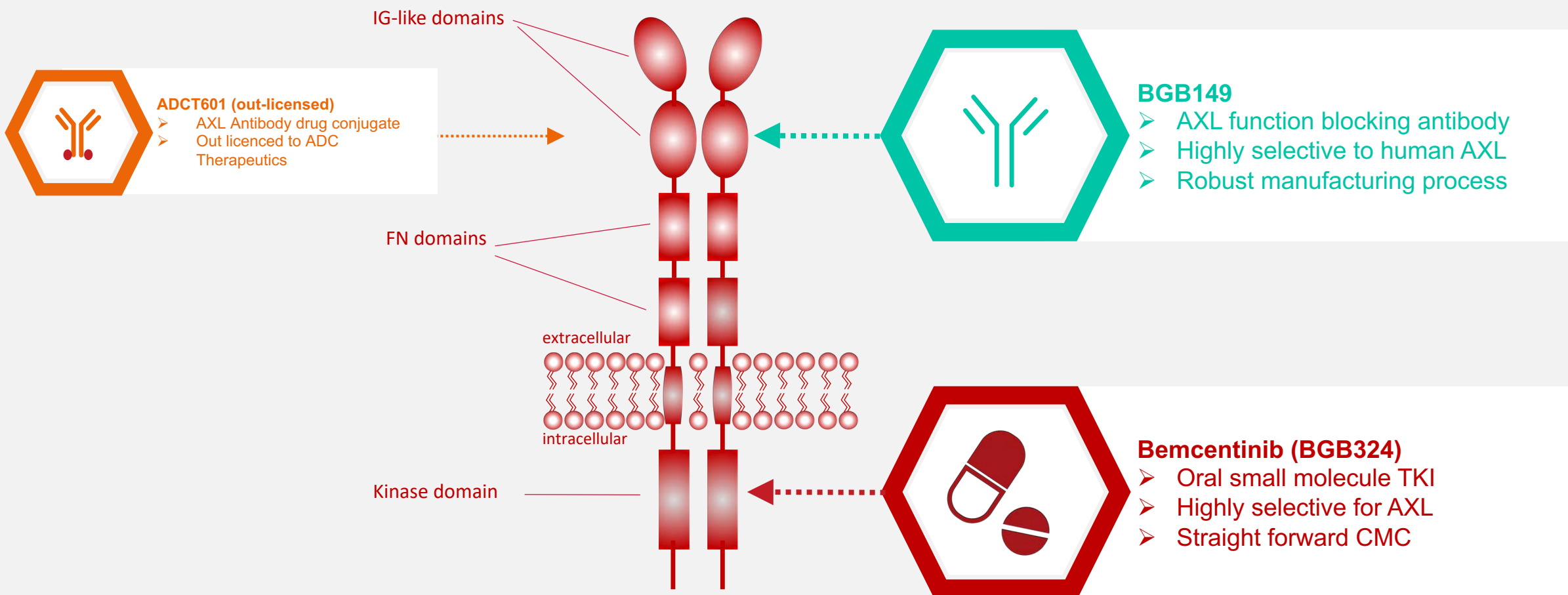
- M1 to M2 macrophage polarisation
- Decreased antigen presentation by DCs
- Immunosuppressive cytokine profile

very low expression under healthy  
physiological conditions (ko mouse  
phenotypically normal)

overexpressed in response to **hypoxia**,  
**immune reaction**, **cellular stress** /  
therapy





overexpression correlates with **worse  
prognosis in most cancers**

# Three AXL-targeting drug candidates in clinical development



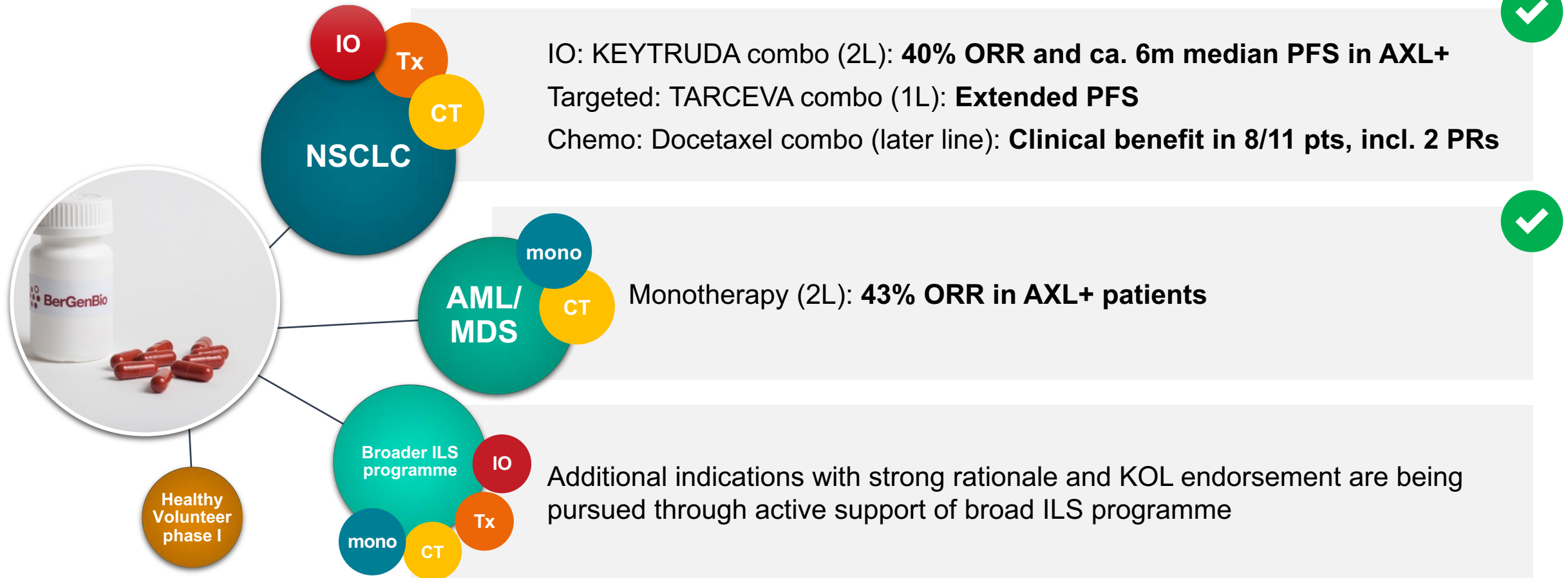
# Clinical development programmes of AXL inhibitors

> 350 patients at 50 sites across Europe and USA

	Preclinical	Phase I	Phase II	Phase III	Status
Selective AXL kinase inhibitors					
Bemcentinib: selective oral small molecule AXL inhibitor					
	NSCLC + KEYTRUDA (2L, IO naïve)	previously treated advanced adenocarcinoma of the lung		 (1)	Stage 1 complete, 40% ORR in AXL+; stage 2 ongoing
	NSCLC + TARCEVA (1L & 2L)	advanced NSCLC with activating mutation of EGFR			Fully recruited, 1 <sup>st</sup> efficacy endpoint met
	NSCLC + docetaxel (later line) <sup>(2)</sup>	previously treated advanced NSCLC			ILS, ongoing – latest update WCLC 2018
	AML single agent + low dose chemo (1L & 2L)	AML or previously treated MDS unfit for intensive chemo			Mono: 43% ORR in AXL+ R/R AML/MDS; decitabine combo completed recruitment
	ILS programme in additional oncology indications <sup>(2)</sup>	Melanoma, mesothelioma, pancreatic, glioblastoma, MDS			Portfolio of ILS, ongoing & in set-up
	Fibrosis – <i>preclinical</i>	IPF, NASH			Pre-clinical work published throughout 2018
BGB149: anti-AXL mAb					
	Healthy volunteers – phase 1a dose escalation	Healthy volunteer SAD			
BGB601: AXL ADC <i>outlicensed</i>					
	Metastatic cancers	First-in-man solid tumours	Out-licensed to 		
Companion Diagnostics Pipeline		Biomarker Discovery	Biomarker Verification	Validation	
Tissue AXL Soluble AXL Additional soluble markers		Correlation with benefit from monotherapy, combo with targeted and immunotherapy			Correlation with efficacy reported



# Summary of Phase II PoC data with bemcentinib (Focus on NSCLC & leukaemia)



# Bemcentinib PoC data summary: Monotherapy and combinations





# Bemcentinib: once-a-day AXL inhibitor

**Highly selective, orally bioavailable small molecule, administered once a day, in phase II clinical trials**

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**Blocks AXL signalling, reverses aggressive tumour traits & counteracts immune escape**

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Clinical PoC in AML and NSCLC as a monotherapy and in combination

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Correlation of clinical efficacy with AXL biomarkers observed

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Excellent clinical safety profile

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Randomised, late stage clinical trials planned to start in H2 2019

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Ref. BGBC003 / NCT02488408

# Acute Myeloid Leukaemia (AML) & Myelodysplastic Syndrome (MDS)

Bemcentinib is being evaluated as a monotherapy and in combination with standard of care to treat AML and high-risk MDS

- ✔ *43% ORR in AXL +ve R/R AML and MDS patients*
- ✔ *chemo combos in 1L ongoing*







# MDS & AML: Disease characteristics

New strategies to treat **older & relapsed/ refractory patients** is an urgent, unmet need

## Myelodysplastic syndromes (MDS) *(pre-leukaemia or smoldering leukaemia)*

Occurs when the blood-forming cells in the bone marrow (the soft inner part of certain bones, where new blood cells are made), become abnormal. This leads to low numbers of one or more types of blood cells.

**~ 40,000 new cases**  
per year (U.S. only)<sup>3</sup>

Most diagnoses made in  
**70s or 80s<sup>1</sup>**

MDS 40% risk of  
developing into  
AML.<sup>4</sup>

## Acute Myeloid Leukaemia (AML)

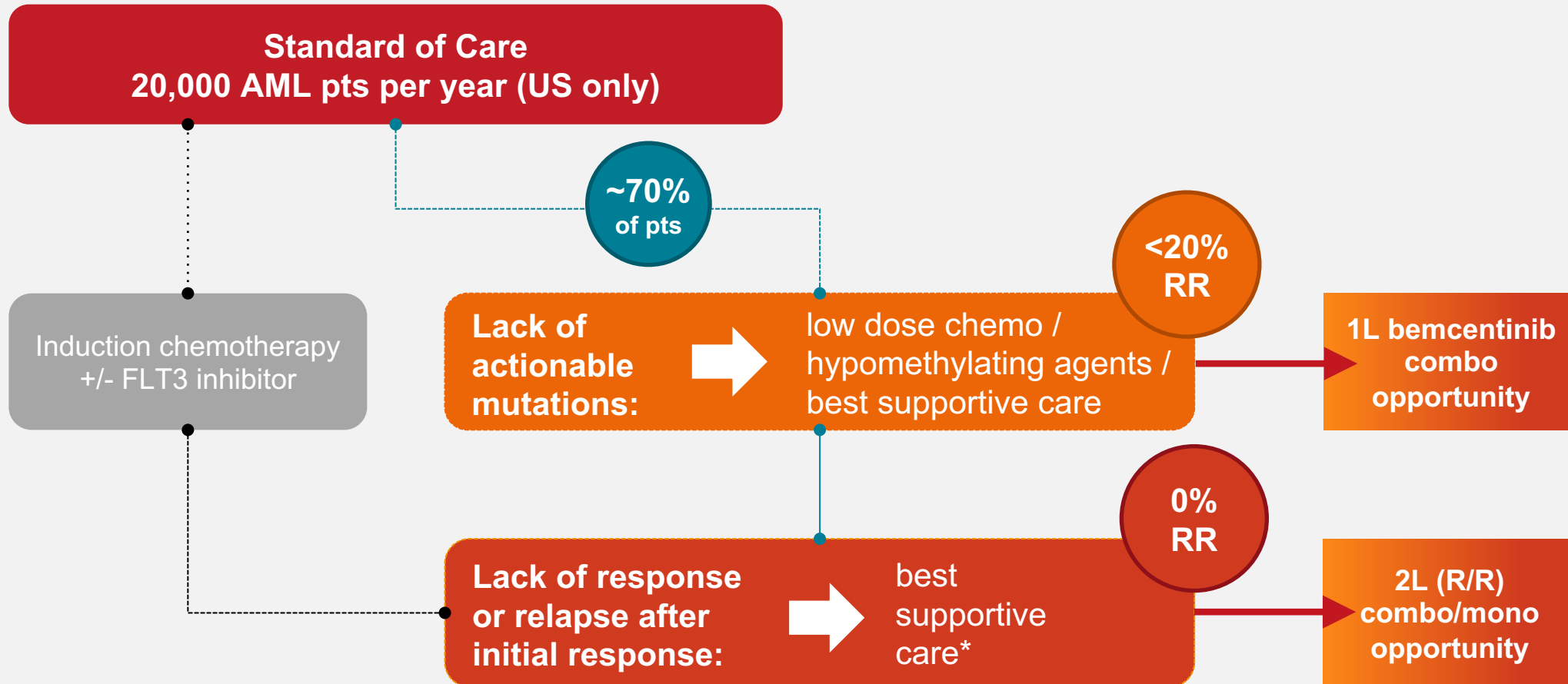
Cancer of the myeloid line of blood cells, characterized by rapid growth of abnormal cells that build up in the bone marrow and blood and interfere with normal blood cells

**~ 20,000 new cases**  
diagnosed and >10,000 deaths (2018, U.S.)<sup>2</sup>

Most common type of  
**acute leukaemia in adults<sup>1</sup>**

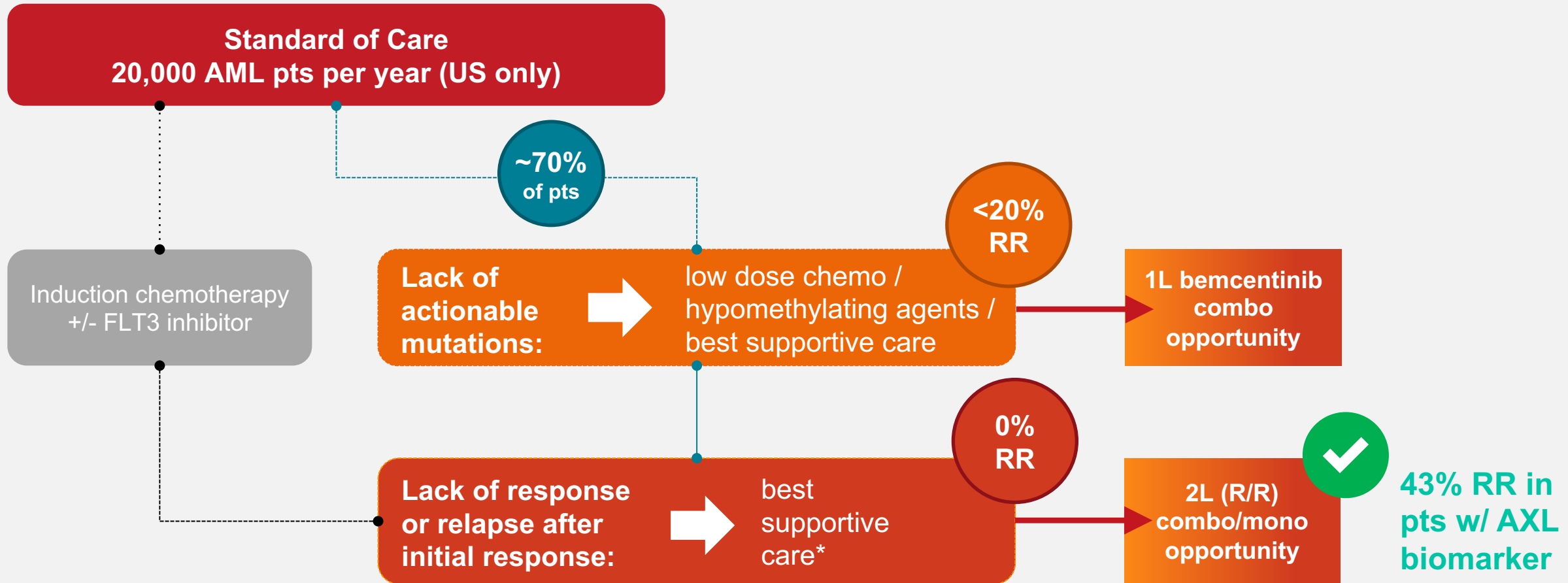


# AML & MDS – difficult to treat malignancies, predominantly elderly frail patient population.



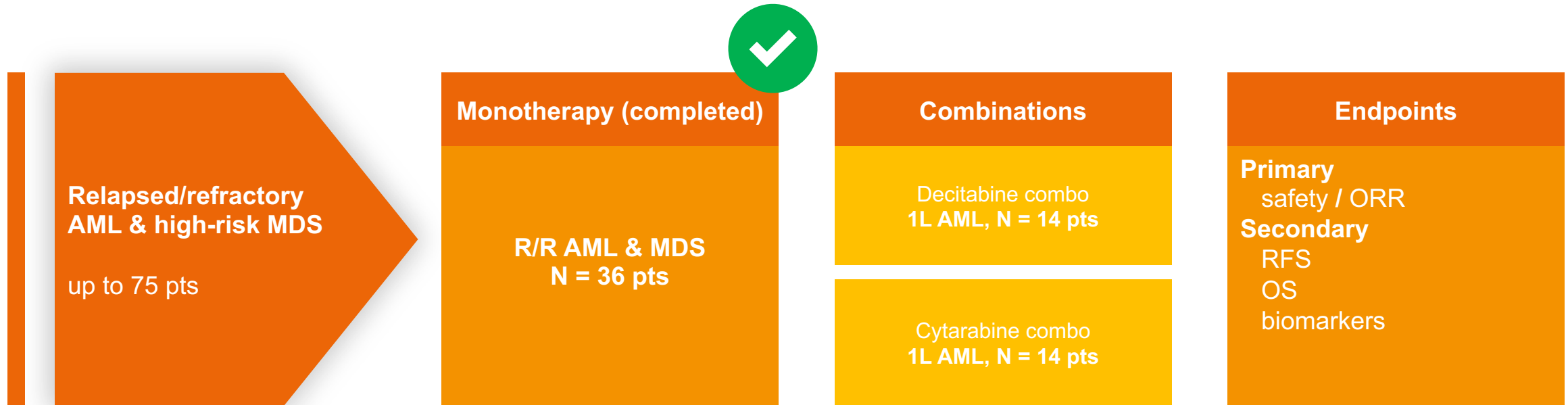


# AML & MDS – difficult to treat malignancies, predominantly elderly frail patient population.





# Phase II trial in AML/high risk MDS: monotherapy and combination with LDCT\*





# Summary results slide and next steps

**Status Jan '19**

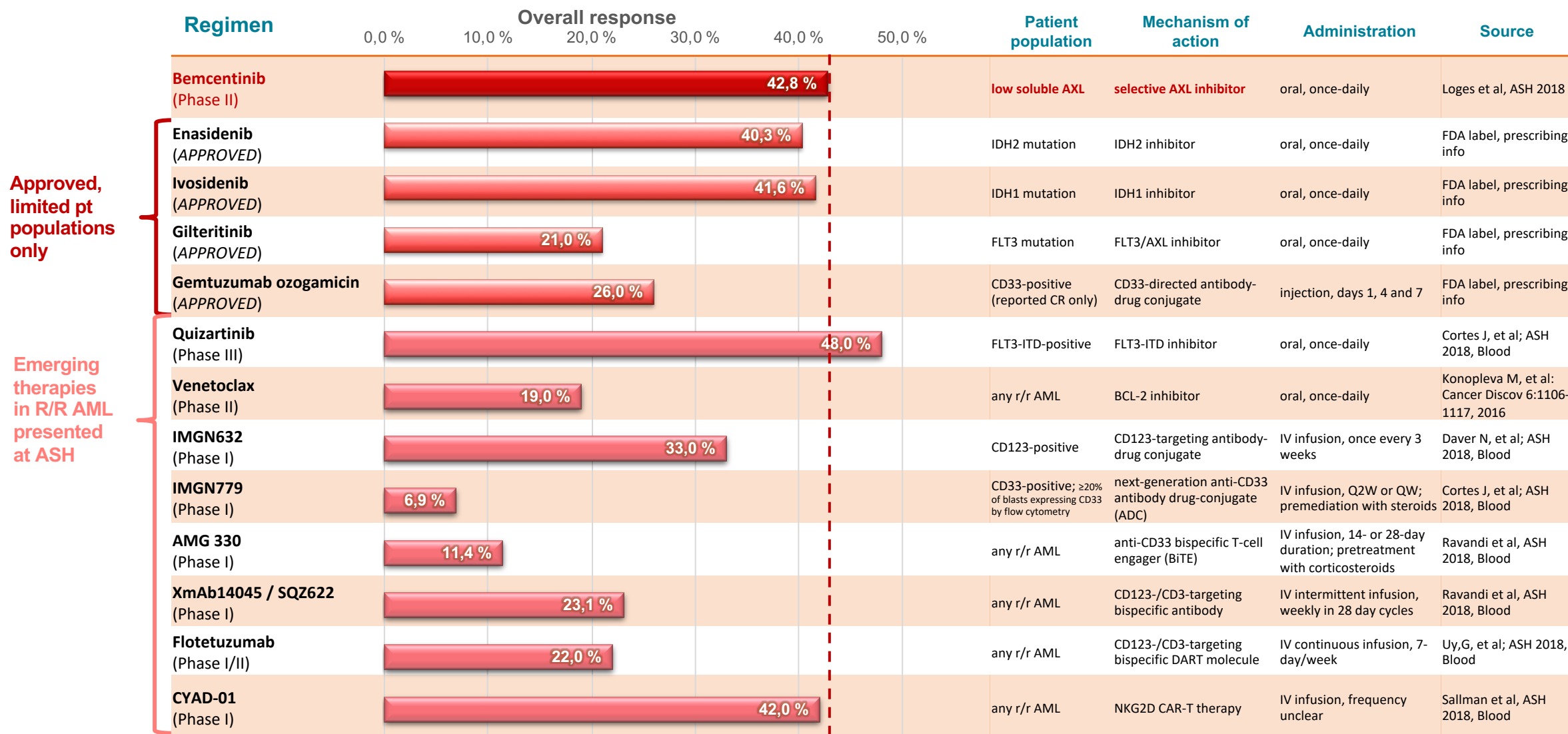
## Summary results

- Monotherapy cohort complete
- 43% ORR in AXL positive patients
- Excellent safety
- Evidence of immune activation

## Next steps

- Chemo combinations ongoing
  - Top line data Q1'19
- Comprehensive analysis anticipated at ASCO/EHA '19

# Monotherapy shows promising efficacy in comparison to approved & emerging regimens



Ref. BGBC008 / NCT03184571

# Bemcentinib in combination with KEYTRUDA in 2L NSCLC

The BGBC008 trial is designed to test whether AXL inhibition can enhance responses to immunotherapy (*KEYTRUDA monotherapy showed 8% response rate\* in previously treated PD-L1 negative NSCLC*), *summary of responses:*

- ✔ **27% ORR in PD-L1 –ve patients**
- ✔ **40% ORR in AXL+ve patients**

Clinical collaboration with Merck & Co. (MSD)



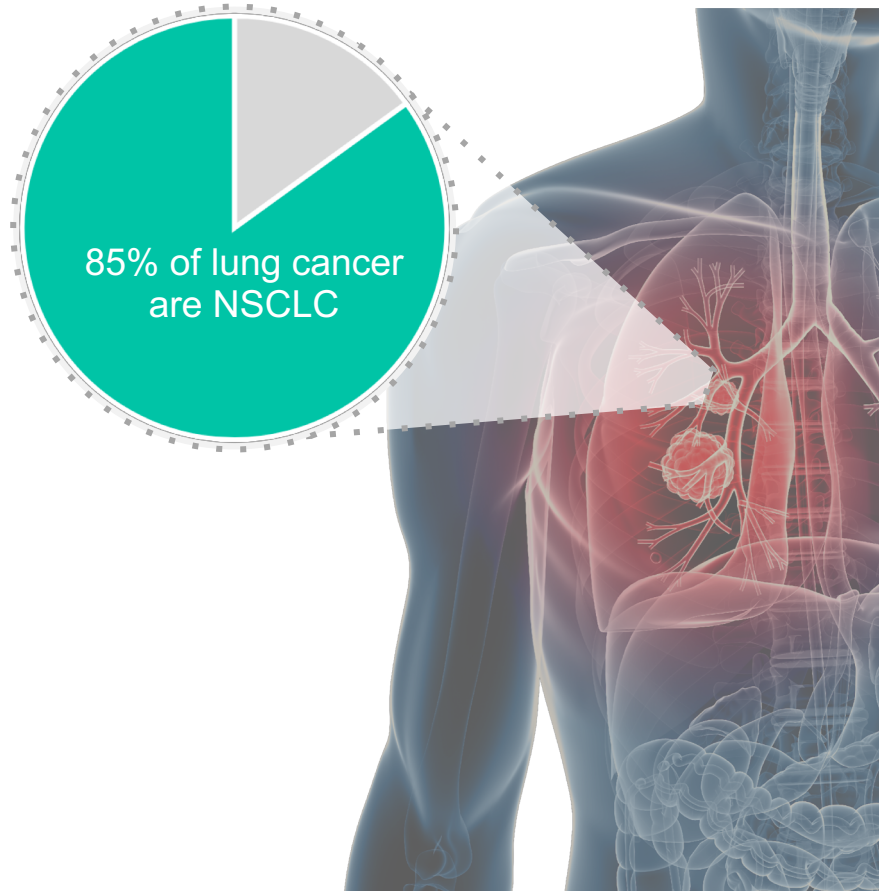
\* Garon, N Engl J Med 2015: Includes any PD-L1 expression







# NSCLC causes more cancer related deaths than breast, colon, pancreas and prostate combined



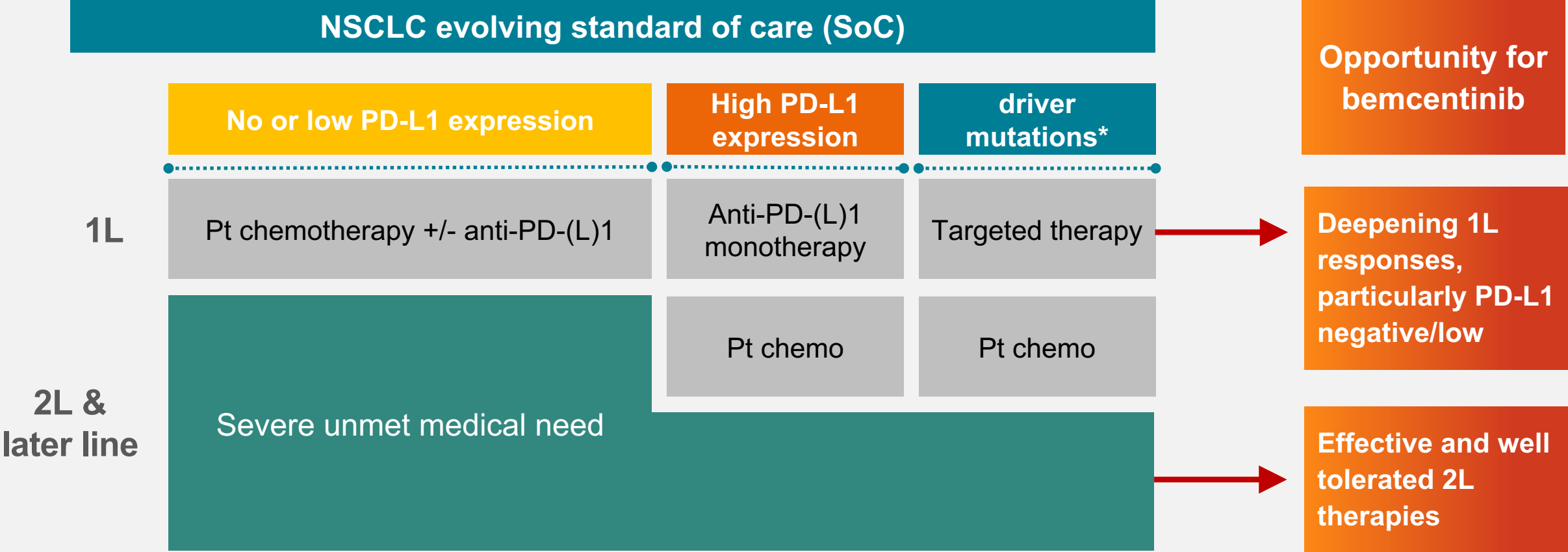
## The largest cancer killer, most patients depend on drug therapy

- 2.09 million new cases of lung cancer diagnosed/yr worldwide, making up 11.6% of all cancer cases<sup>1</sup>
- 1.76 million lung cancer deaths/yr worldwide<sup>1</sup>
- In the U.S, 5-year survival rate is approximately 18.6%, and **4.7%** in patients with distant metastases<sup>2</sup>

**Non-small cell lung cancer is the most common type of lung cancer, making up 80-85% of lung cancers**

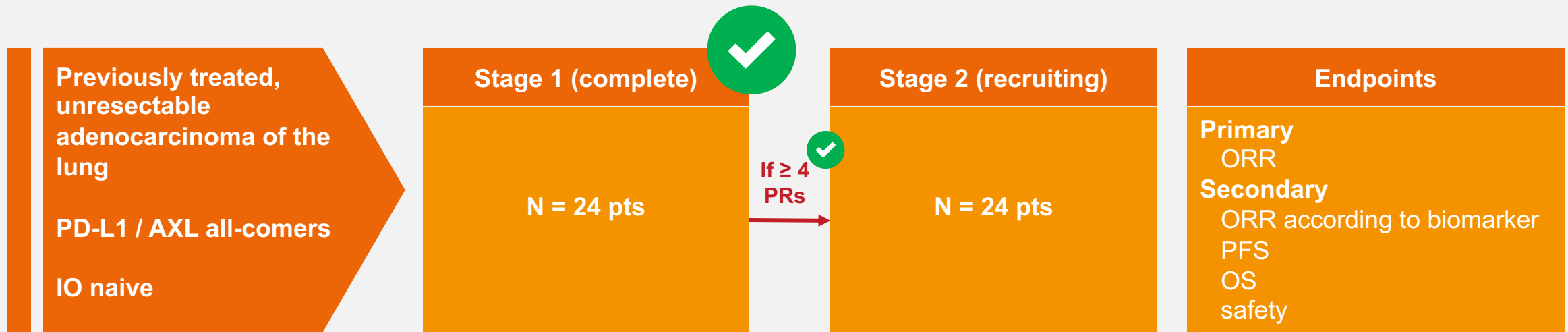


# Rapidly emerging SoC creates opportunities for novel effective, chemo free regimens



\* Mutations / rearrangements with available targeted therapies such as EGFR and ALK

# Phase II 2L NSCLC study of bemcentinib with KEYTRUDA



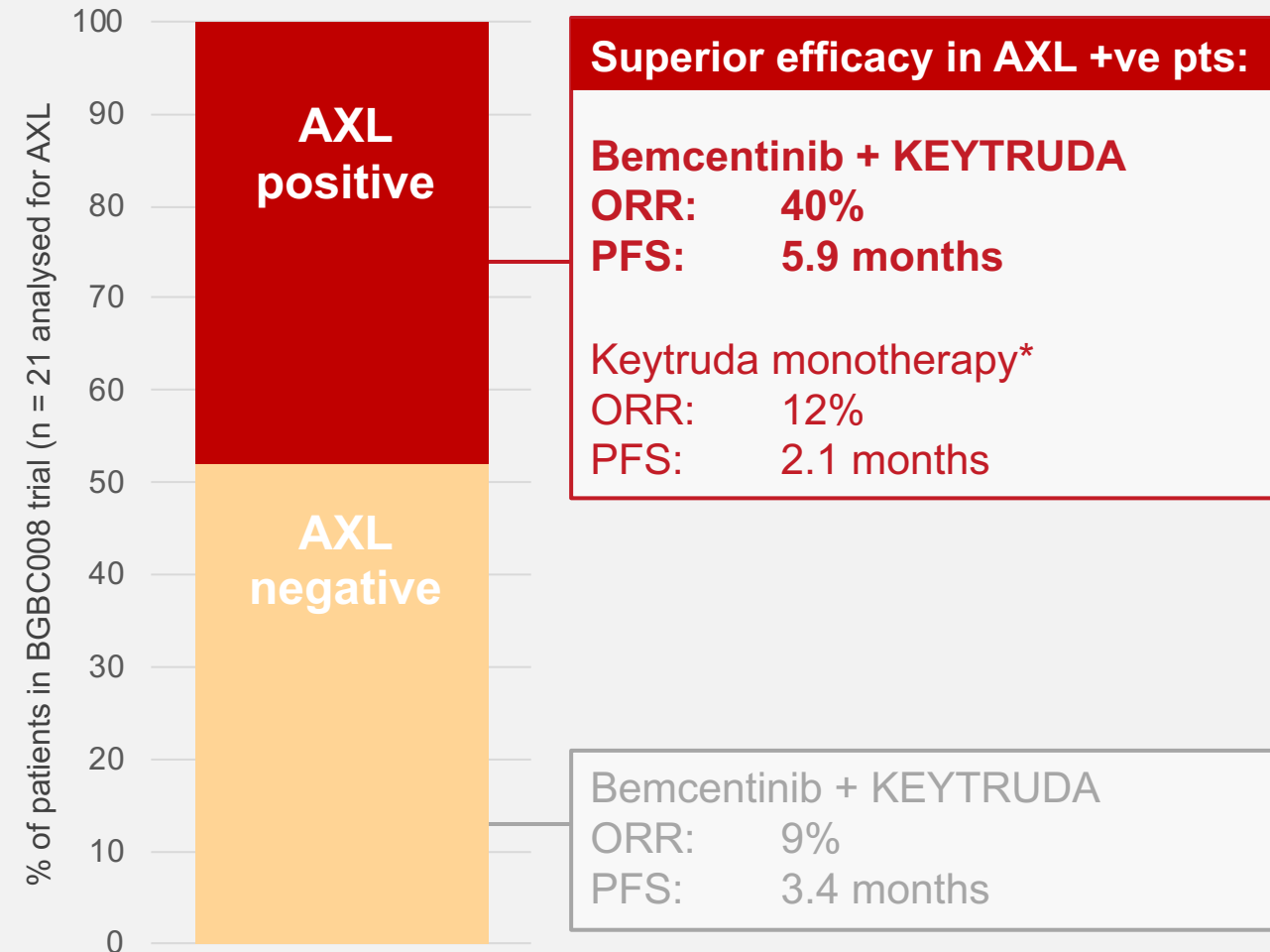
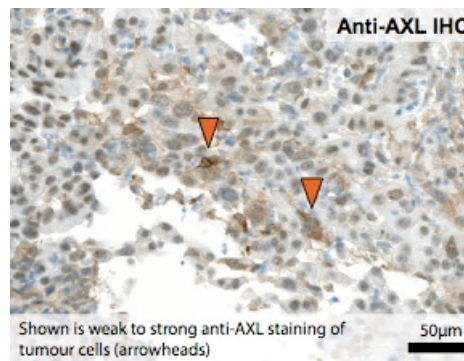
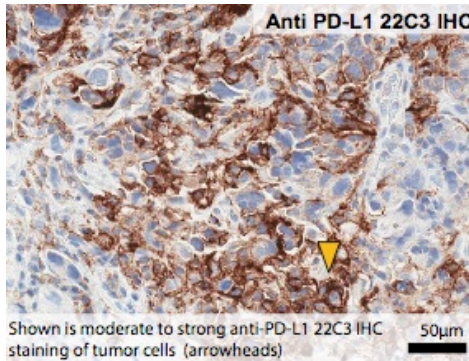
## Key objectives

- Evaluate safety of the combination and response to treatment with the combination
- Characterise patients by PD-L1 and AXL status
- Evaluate efficacy of patients by biomarker status, and assess predictive qualities of biomarkers
- Assess survival measures in patients by biomarker status

# Response per biomarker expression

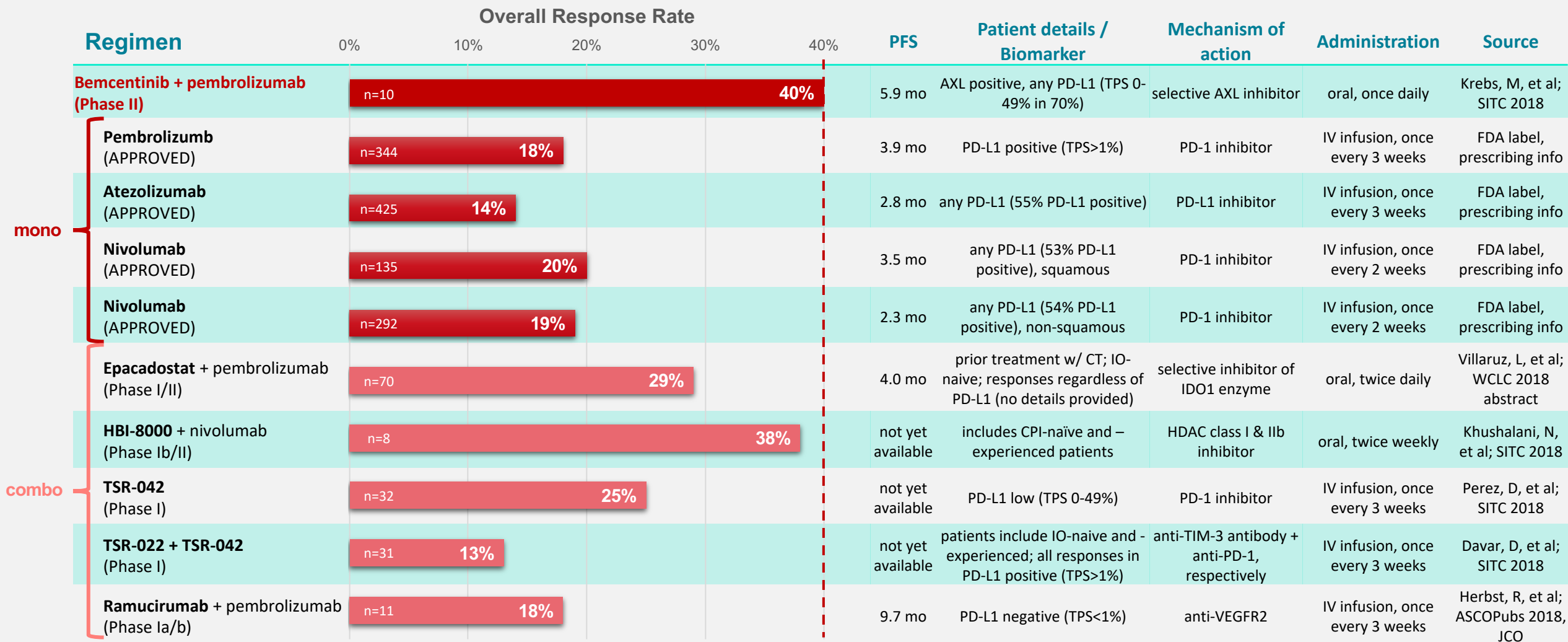
## Analysis of biomarker expression in the BGBC008 trial revealed:

- ✓ Appr. **half of patients were AXL positive** (10 out of 21 analysed for AXL)
- ✓ The **vast majority of patients did not express high levels of PD-L1**, the biomarker for KEYTRUDA monotherapy efficacy





# Promising efficacy in comparison to approved monotherapy and emerging combinations\*

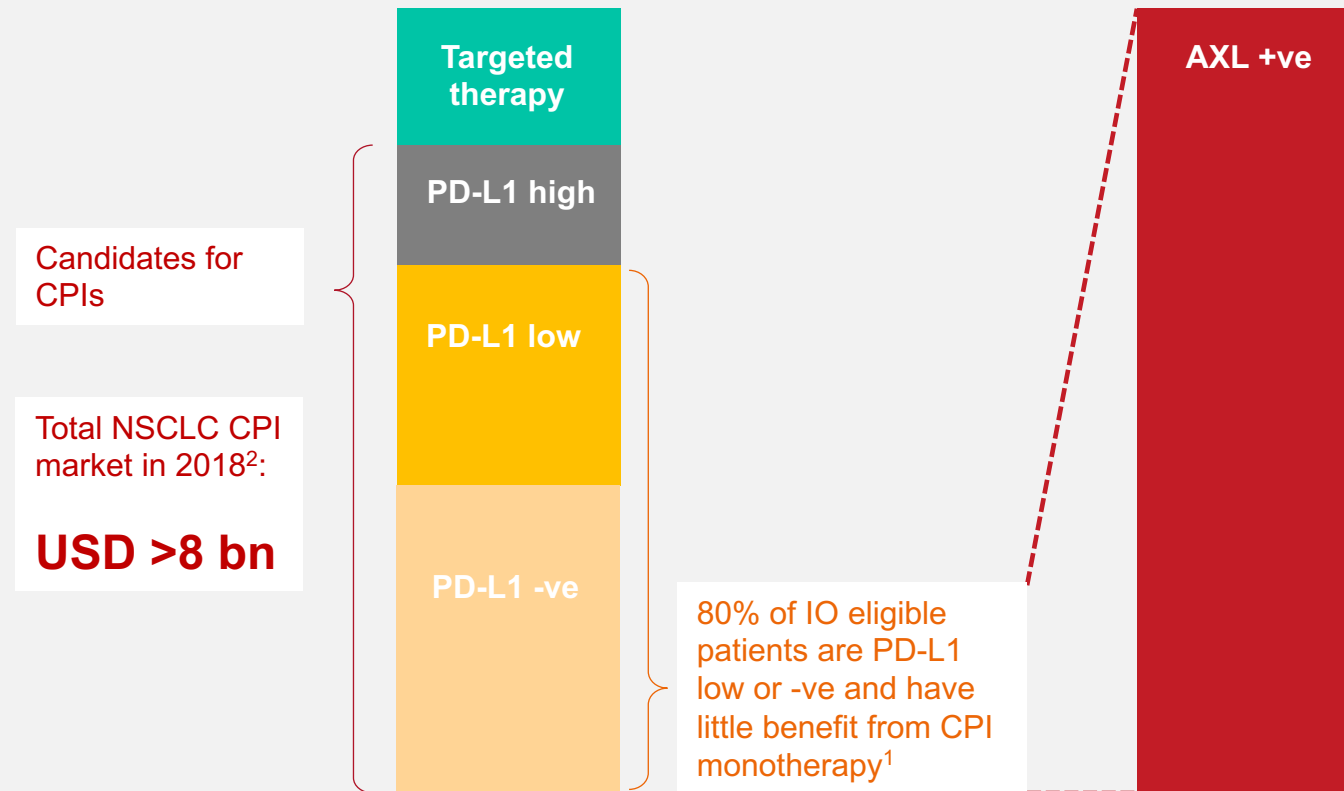




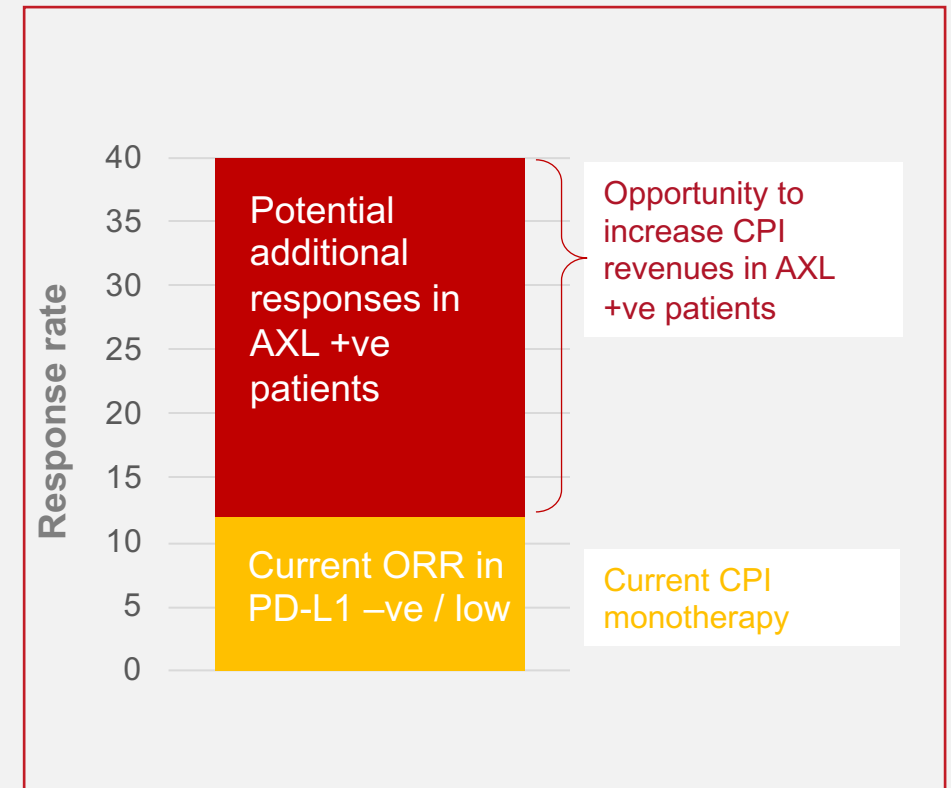
# The majority of NSCLC patients are eligible for CPIs, but only few respond: novel combination agents will drive CPI differentiation & multi-billion market opportunity

Late stage NSCLC  
pts / yr (US only)

**135,000**



of BGBC008 patients found positive for AXL, in these patients response was tripled compared to monotherapy





# Summary results and next steps

## Status Jan '19

### Summary results

- 1st efficacy endpoint met
- 40% ORR and 6m PFS in AXL positive patients
- 27% ORR in PD-L1 -ve patients

### Next steps

- Second stage ongoing
- **H1 '19**
  - Top line OS for stage 1
  - Preliminary ORR per biomarker stage 2
- **H2 '19**
  - Final efficacy & biomarkers stage 1 + 2
  - PFS

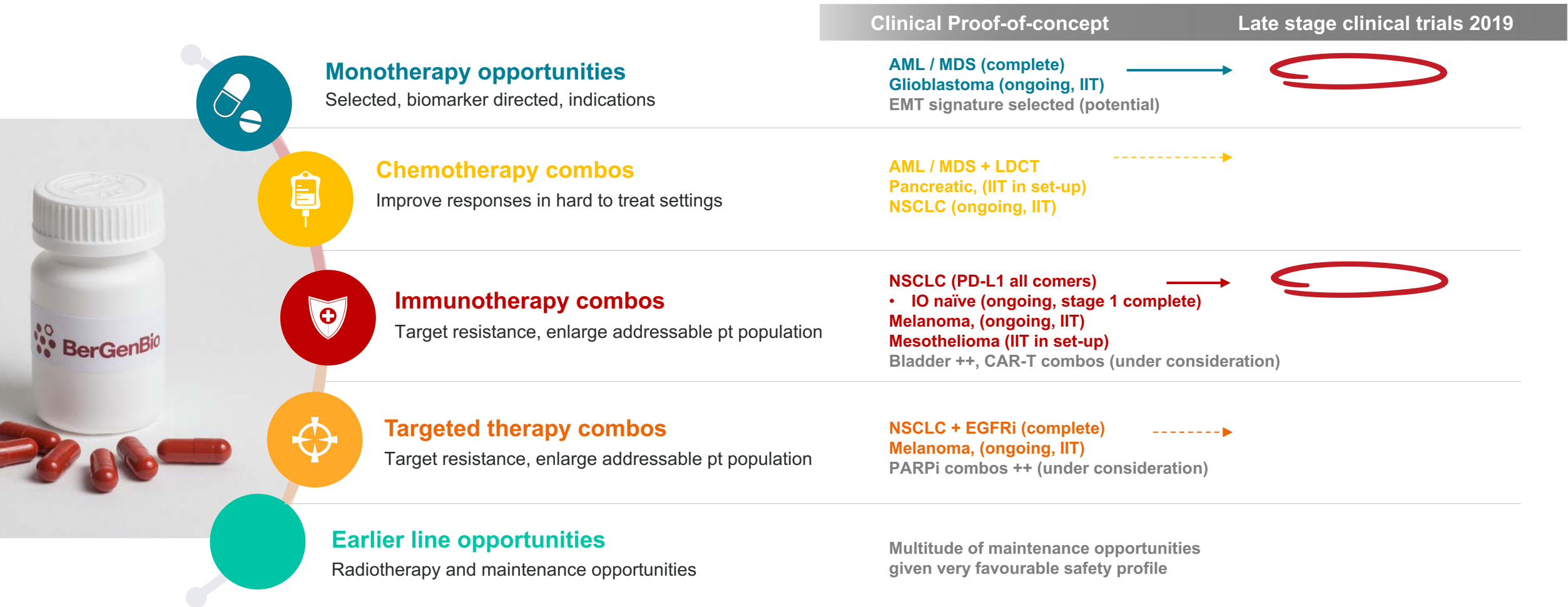


# Clinical development opportunities for bemcentinib



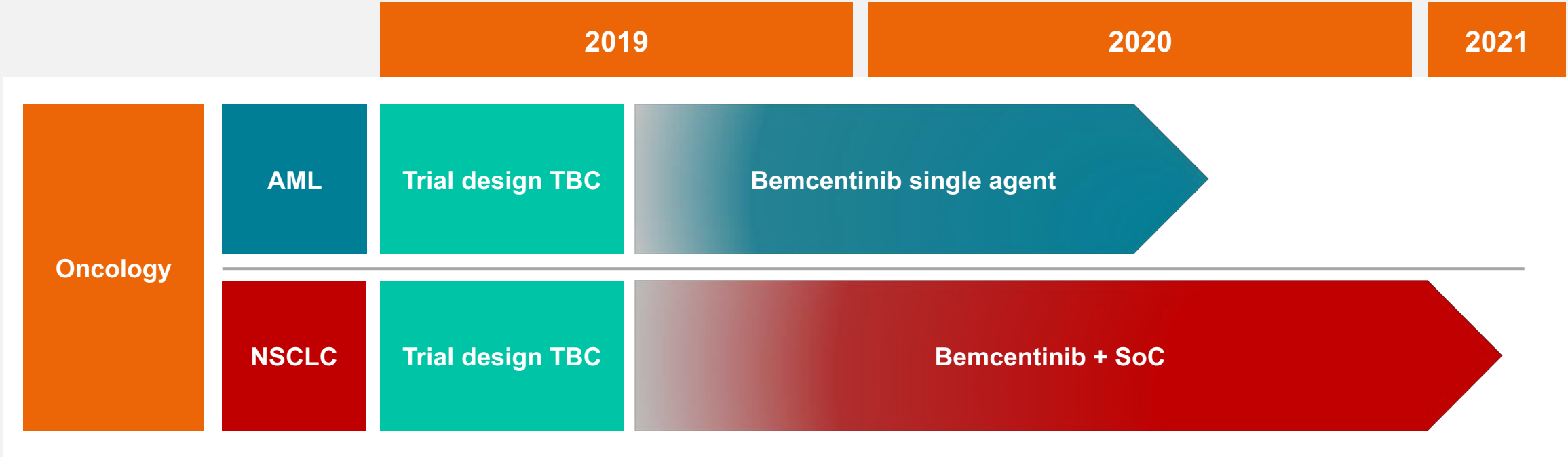


# Clinical Development opportunities for bemcentinib





# Data generated provides strong rationale for late stage clinical trials to start in 2019\*



# BGB149 – a monoclonal anti-AXL antibody





# BGB149: Anti-AXL monoclonal antibody

## Phase I clinical trial initiated January 2019

### Functionally blocking humanised monoclonal antibody

#### Binds human AXL, blocks AXL signalling

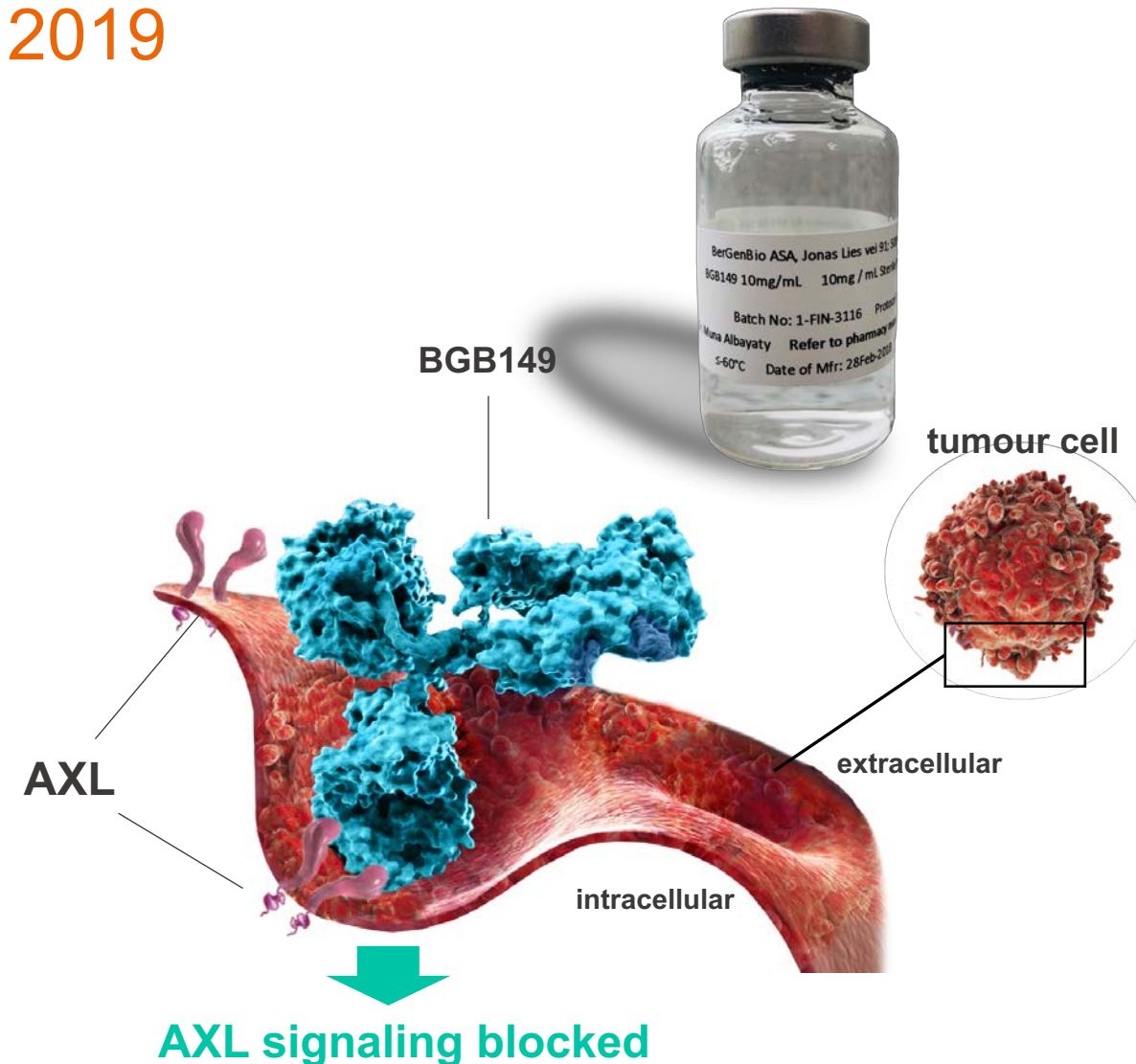
High affinity (KD: 500pM), Anti-tumour efficacy demonstrated *in vivo*

Robust manufacturing process established, 18 months stability

First-in-human healthy volunteer Phase I study initiated

- Up to 36 subjects
- Safety, PK/PD

First-in-patient trial expected in H2 2019



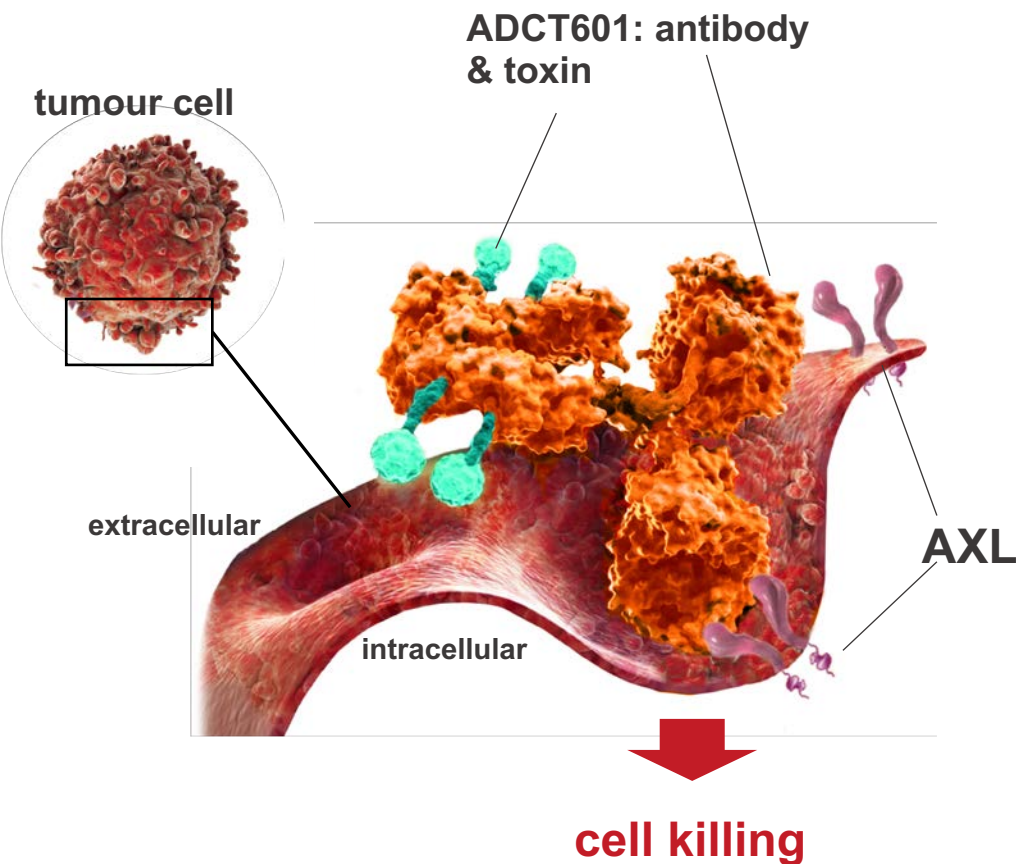
# ADCT-601 – AXL ADC





# BGB601/ADCT-601: Anti-AXL ADC

## Phase 1 in solid tumours started January 2019



### Antibody Drug Conjugate (ADC)

Targets human tumour AXL, induces cell death when internalised

Potent and specific anti-tumour activity demonstrated preclinically<sup>1</sup>

### First-in-human Phase I study initiated in Jan 2019

- Solid tumours
- Up to 75 patients
- Safety, PK/PD, preliminary efficacy

Based on anti-AXL antibody BGB601 licensed from BerGenBio

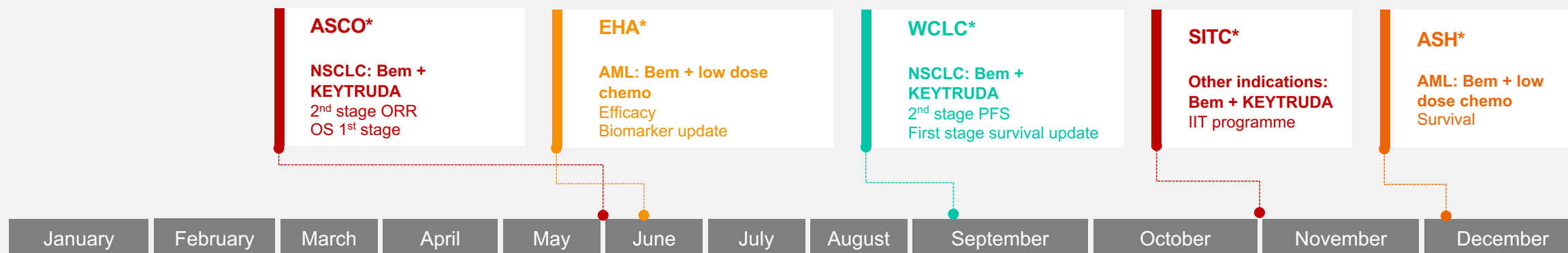


# Near term goals and news flow





# Anticipated clinical data readouts and operational milestones for 2019



\* expected

## Q1

- 1L/2L AML chemo combo top line data
- Complete recruitment stage 2 bem + KEYTRUDA (NSCLC)

## Q2/Q3

- **Initiate randomised programme**
- Complete Phase 1 BGB149

## Q4

- Initiate first-in-patient trials BGB149

# Upcoming company news flow and value creating catalysts

Strategic priority		Goals	
<b>Late stage clinical trials with bemcentinib</b>	H2 2018	Clinical PoC monotherapy AML	✓
	H2 2018	Clinical PoC combo in NSCLC	✓
	H1 2019	Clinical PoC combo in AML	
	H2 2019	Start late stage clinical programme	
	H2 2020	Interim read-out late stage clinical programme	
<b>Develop Companion Diagnostics</b>	H2 2018	Identify candidates that correlate with efficacy	✓
	H2 2020	Validate candidates in late stage clinical programme	
	H2 2021	Clinical assay developed	
<b>BGB149 anti-AXL antibody programme</b>	H2 2018	Initiate first-in-man phase I trial	✓
	H2 2019	Initiate first-in-patient phase Ib trial	
	H2 2020	Interim readout	
<b>Maximise value for bemcentinib</b>	H1 2019	Initiate pipeline opportunities for bemcentinib via ISTs	✓

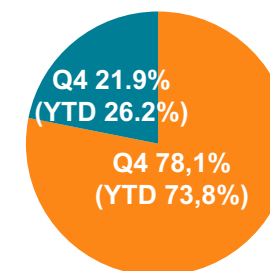
# Financial review: Good financial position and cost control

Rune Skeie  
CFO

# Key financial figures

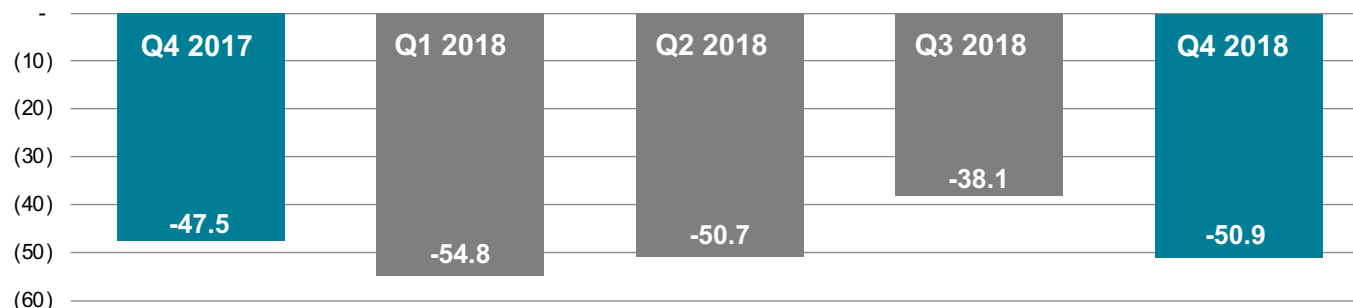
(NOK million)	Q4 2018	Q4 2017	FY 2018	FY 2017
Operating revenues	2.3	0	2.3	0
Operating expenses	53.2	47.5	196.9	183.7
Operating profit (loss)	-50.9	-47.5	-194.5	-183.7
Profit (loss) after tax	-51.1	-47.6	-191.7	-182.2
Basic and diluted earnings (loss) per share (NOK)	-0.93	-0.96	-3.60	-4.01
Net cash flow in the period	-37.8	-28.8	-9.9	208.5
Cash position end of period	360.4	370.3	360.4	370.3

## Operating expenses Q4 2018



■ R&D ■ Administration

## Operating profit (loss) million NOK

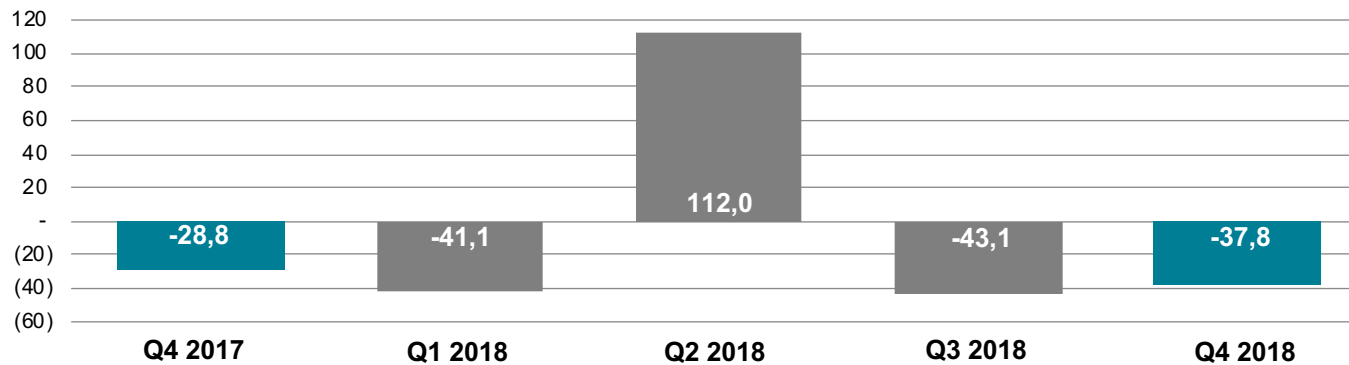


- Effective organisation
- 78.1% (YTD 73.8%) of operating expenses in Q4 2018 attributable to Research & Development activities

- Q4 18 operating loss reflecting level of research and development activities in the quarter
  - Revenue NOK 2.3 million, licence revenue triggered by pre-clinical milestone (ADCT-601)
  - Stage 2 of NSCLC combination with Keytruda re-opened in Q4 18 and ongoing (mandatory safety review in Q3 18)

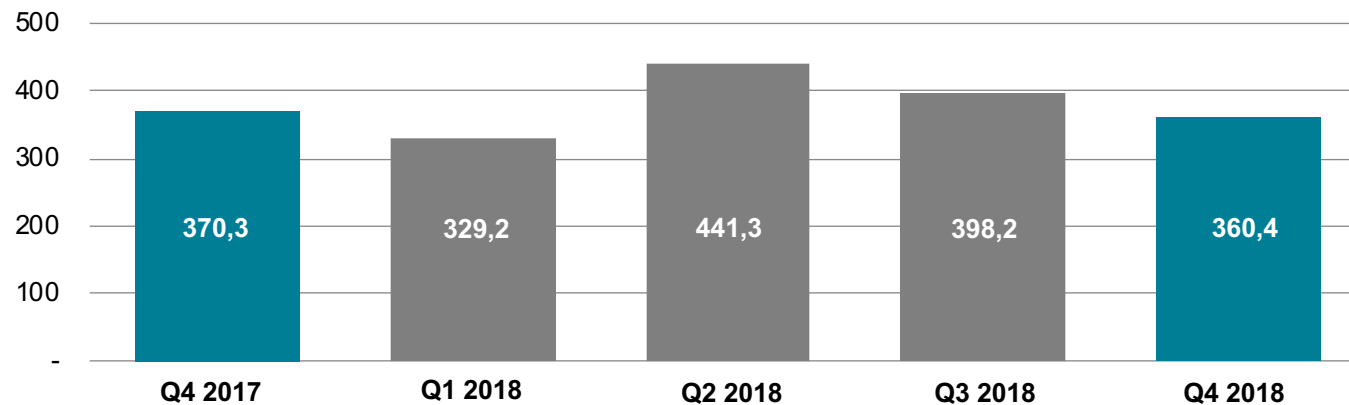
# Cash flow and cash position

## Cash flow (mill NOK)



- Private placement Q2,18 strengthened cash position - gross funds raised NOK 187.5m
- Quarterly cash burn average 2018 at NOK 46.7 million

## Cash position (mill NOK)



- Cash position gives runway to deliver key clinical read outs from ongoing clinical studies
- Cash runway into 2020 based on current burn rate

# Full year 2018 highlights

## **Strong Phase II PoC clinical data readouts with bemcentinib:**

All operational milestones met with data presented at international clinical congresses

## **Data paving the way to late stage clinical trial programme in 2019:**

Targeting monotherapy and combination opportunities in AML/MDS and NSCLC

## **Pipeline opportunities pursued to complement key internal programmes:**

Investigator-led studies broaden oncology applications; rationale in fibrosis

## **Bemcentinib biomarker programme progressed:**

Efficacy correlation with AXL reported in clinical trials