Aptose Corporate Event in Concurrence With 2021 EHA Congress

June 11, 2021

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PRECISION ONCOLOGY FOR THERAPIES OF TOMORROW

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Aptose is a Clinical Stage Biotech Company Developing a Pipeline that Addresses Unmet Needs in Hematologic Malignancies

- Developing 1st-in-class precision medicines for the treatment of life-threatening hematologic cancers
- Agents suppress validated leukemia targets to serve the needs of patients with deep R/R disease
- Multiple assets addressing multiple cancer indications for optionality and value creation

Luxeptinib

(CG-806 FLT3/BKT Inhibitor)

- ***Ph 1 single agent activity in AML***
- ***Ph 1 leading indicators of clinical activity in B-cell cancers***
- Hits WT/Mutant ***FLT3*** and ***BTK*** kinases
- Hits multiple validated cancer targets

APTO-253

(MYC Program)

- ***Phase 1*** dose escalating trial in ***AML/MDS***
- Hits notable ***MYC*** oncogene
- Inhibits MYC protein expression
- Stabilized G-quadruplex in MYC gene

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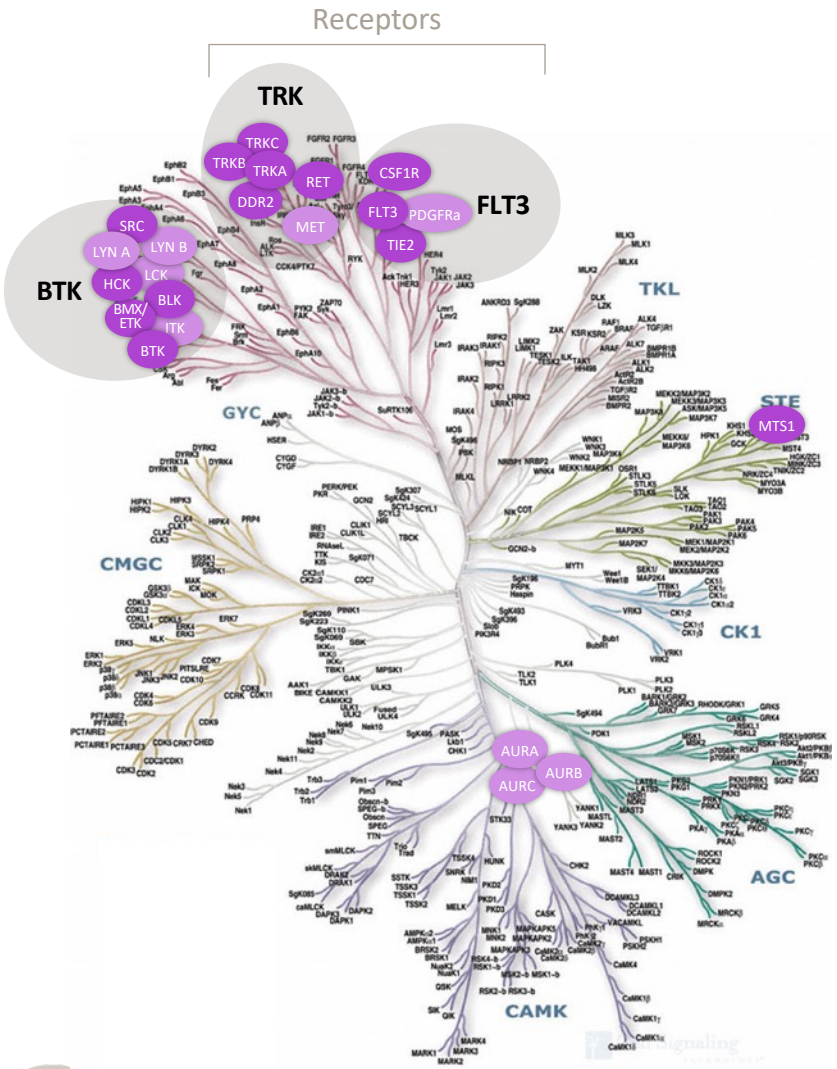
Luxeptinib (CG-806)

1st-in-Class Oral Kinase Inhibitor

Mutation Agnostic FLT3 Inhibitor

Mutation Agnostic rBTK Inhibitor

Luxeptinib “Cluster-Selective Kinase Inhibitor”: Potently and Selectively Inhibits Clusters of Related Kinases



Unique Kinome Targeting

Selectively inhibits clusters of kinases

Inhibits **FLT3: WT and all mutant forms**

Inhibits **BTK: WT and all mutant forms**

Only agent to potently inhibit the validated **BTK** and **FLT3** targets

Simultaneously suppresses **multiple oncogenic signaling pathways**

- Direct: FLT3, BTK, ITK, SYK, LYN, TRK, CSF1R, PDGFRα
- Downstream Pathways: STAT, ERK, MAPK, AKT, MYC

Robust Safety

Avoids kinases that negatively impact safety

NOT a “dirty” kinase inhibitor

Impact

Potential broad activity across hematologic malignancies

May treat B-cell cancer patients failing other BTK inhibitors

May treat AML cancer patients failing other FLT3 inhibitors

May avoid rapid emergence of drug resistance

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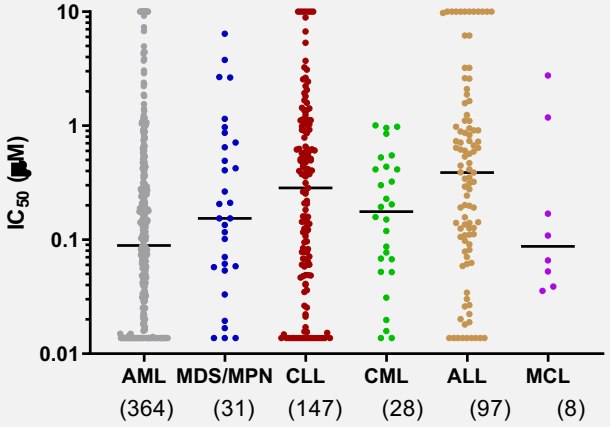
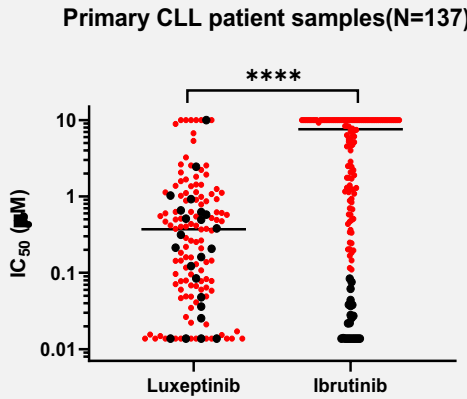
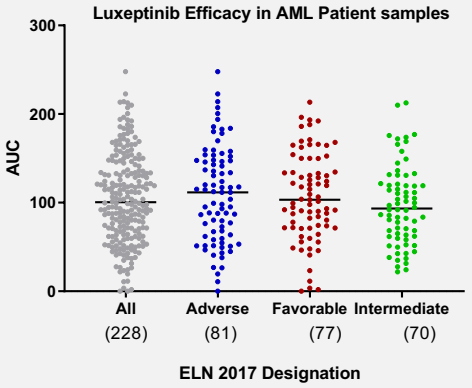
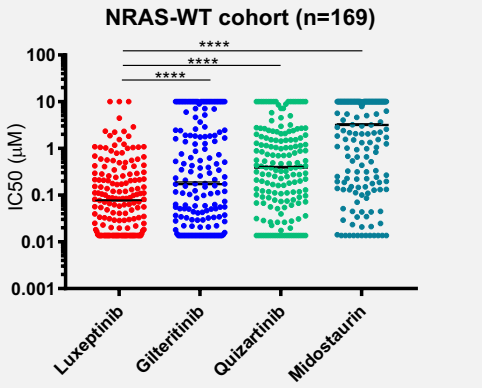
Luxeptinib Broad Potency Across 675 Hematologic Cancer Patient Samples: Mechanistically Distinct, Resilient to Mutations, Combines Well

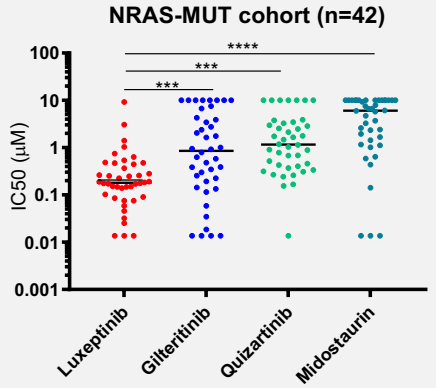




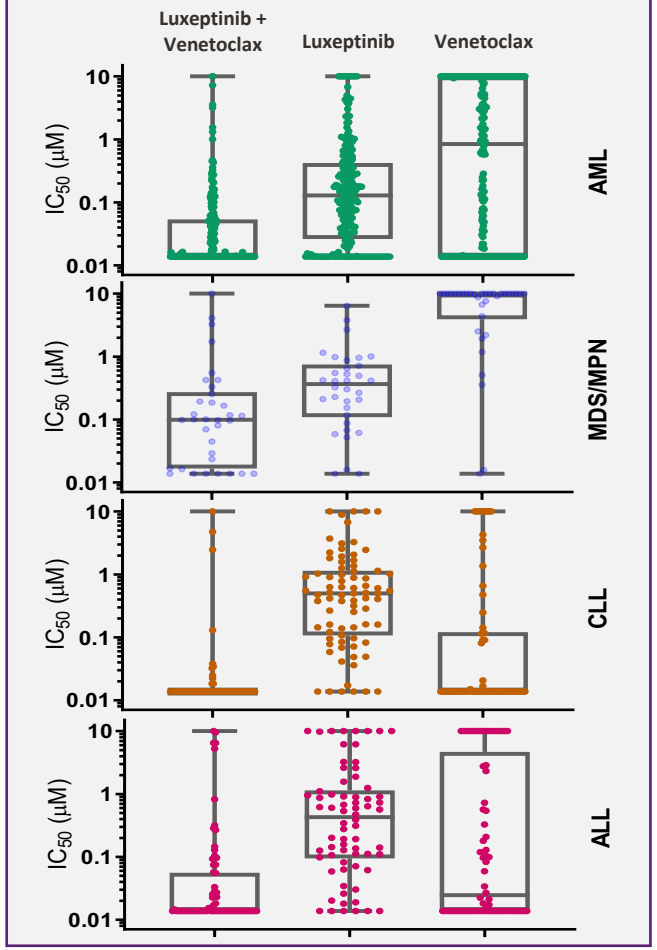




	Luxeptinib	Gilteritinib	Quizartinib	Midostaurin
Median	0.0768	0.171	0.389	3.19



	Luxeptinib	Gilteritinib	Quizartinib	Midostaurin
Median	0.180	0.857	1.16	6.03



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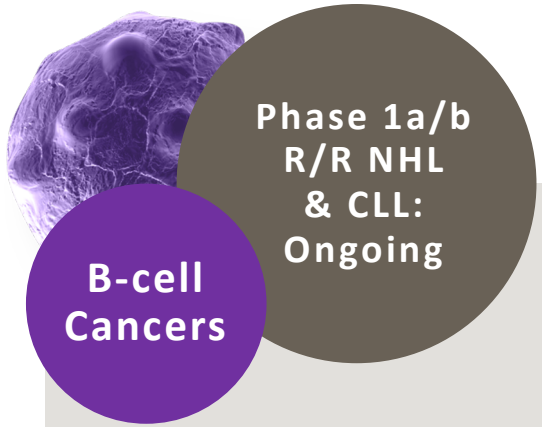
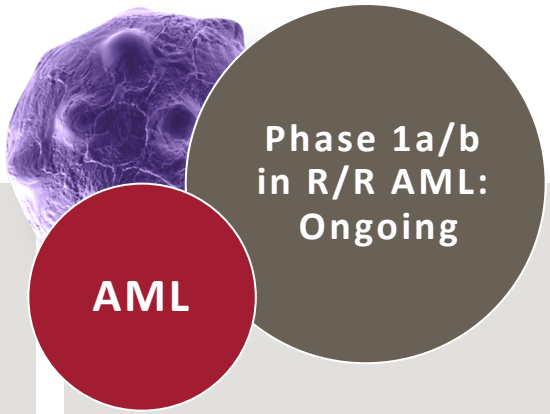
Luxeptinib Phase 1 Clinical Development Plan for Patients with B-cell Malignancies and AML



- Dose escalation began at 150mg BID dose level 1 and currently at 750mg BID dose level 5
- Seek to define safety, tolerance, PK, PD and RP2D
- Seek to inhibit phospho-BTK, induce lymphocytosis, observe responses in B-cell cancer patients



- Dose escalation began at 450mg BID dose level 1 and currently at 750mg BID dose level 3
- Seek to define safety, tolerance, PK, PD and RP2D
- Seek to inhibit phospho-FLT3, decrease PB blast counts and observe responses in AML patients

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Luxeptinib (CG-806)

Phase 1a/b Trial for Patients with B-cell Cancers

Luxeptinib Phase 1a/b Clinical Trial Underway: Patients with Heavily Pretreated B-Cell Malignancies

Patient Population

Relapsed or refractory CLL/SLL & NHL who **failed or are intolerant** to 2 or more lines of established therapy, or for whom no other treatment options are available

Dose Escalation Phase

- Patients administered **oral capsules**
- **Twice daily** on a **28-day cycle**
- Plan to perform 6 dose levels
- Planned expansion cohorts
- **Accelerated titration** design

Development Plan for Severe Unmet Needs in B Cell Tumors

CLL Patients Resistant or Intolerant to:

- Covalent BTK inhibitors (ibrutinib)
- BCL2 inhibitors (venetoclax)
- Anti-CD20 therapy (rituximab)
- PI3K inhibitors (idelalisib)
- Cytotoxic agents
- Non-covalent BTK inhibitors

NHL Patients with Unmet Needs

- Richter's Transformation
- Tx-refractory DLBCL
- Tx-refractory FL

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Luxeptinib Phase 1a/b Clinical Trial in Patients with Heavily Pretreated B-cell Cancers: Now Dosing Cohort 5 (750 mg BID)

Cohort	Dose	Status
1	150 mg BID	Completed
2	300 mg BID	Completed
3	450 mg BID	Completed
4	600 mg BID	Completed
5	750 mg BID	*Ongoing*
6	900 mg BID	Planned

To date observed all three leading indicators of clinical activity:

a. ***Target engagement****: dose-dependent inhibition of phospho-BTK*

b. ***Treatment-related lymphocytosis*** *in patients presenting with classic CLL*

c. ***Modest tumor reduction*** *across different B-cell malignancies (FL, CLL, SLL)*

Currently treating patients at fifth dose level (750mg BID)

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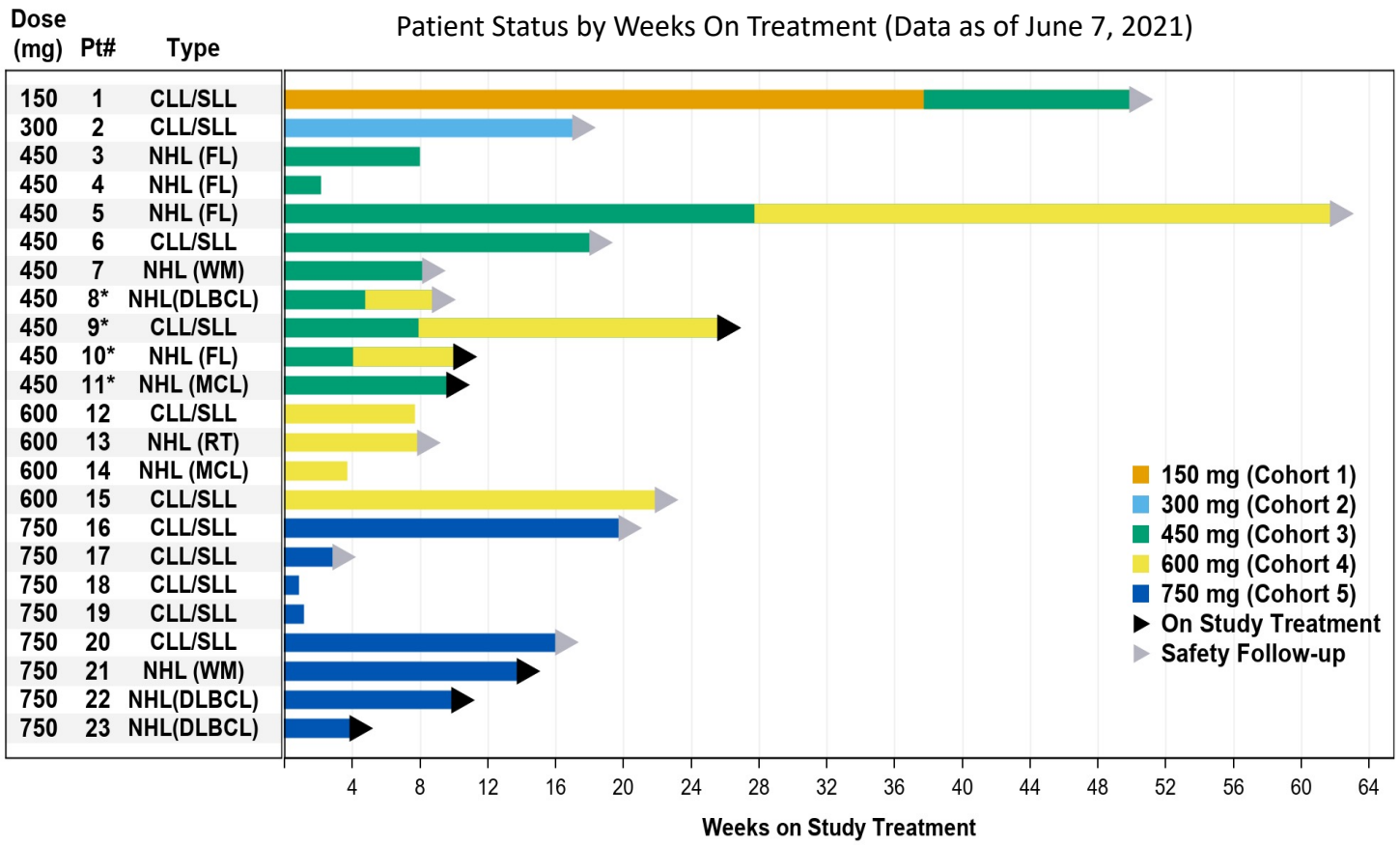
Swimmers' Plot and Demographics of Patients with Relapsed or Refractory B-Cell Malignancies Treated with Luxeptinib



A diversity of heavily pretreated patients are being treated at the expanded 750mg dose and backfilled at the 450mg dose which allows for subsequent intra-patient updosing.

Patient Demographics	Cohorts 1 to 5 (N=22)*
Median Age (Range), Years	**64.5 (55, 84)**
Sex, N (%)	
Male	**13 (59.1%)**
Female	**9 (40.9%)**
Ethnicity, N (%)	
Not Hispanic or Latino	**18 (81.8%)**
Hispanic or Latino	**3 (13.6%)**
Not Reported	**1 (4.5%)**
Race, N (%)	
White	**20 (90.9%)**
Black or African American	**2 (9.1%)**
ECOG Score, N (%)	
0 -Normal activity	**11 (50.0%)**
1 -Symptoms, but ambulatory	**11 (50.0%)**
Disease Type, N (%)	
CLL/SLL	**11 (50.0%)**
NHL	**11 (50.0%)**
Relapsed or Refractory, N (%)	
Relapsed	**11 (50.0%)**
Refractory	**4 (18.2%)**
Both Relapsed and Refractory	**7 (31.8%)**
Intolerant to Prior Therapy, N (%)	**10 (45.5%)**
Median Number of Lines of Prior Therapy (Range)	**3 (1, 12)**
Chemotherapy, N(%)	**20 (90.9%)**
Radiation, N(%)	**4 (18.2%)**
Targeted and Immunotherapy, N (%)	
BTK-Inhibitor (ibrutinib, acalabrutinib, AVL-292)**	**12 (54.5%)**
Anti-BCL2 (venetoclax)	**6 (27.3%)**
PI3K-Inhibitor (idelalisib, duvelisib)	**5 (22.7%)**
Proteasome Inhibitor	**2 (9.1%)**
Other Kinase Inhibitor	**1 (4.5%)**
Antibody	**22 (100%)**
Steroid	**9 (40.9%)**
Immunomodulatory Agent	**5 (22.7%)**
Cellular	**2 (9.1%)**
Other	**2 (9.1%)**

*Data-cut date: Apr 22, 2021

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AE and Safety Profile of Patients with Relapsed or Refractory B-Cell Malignancies Treated with Luxeptinib

Treatment Emergent Adverse Events				
Preferred Term	Cohorts 1 to 5 (N=22)*			
	All TEAE		Related TEAE	
	Any Grade, N** (%)	Grade 3-4, N (%)	Any Grade, N(%)	Grade 3-4, N (%)
Nausea	7 (31.8%)	0	6 (27.3%)	0
Vomiting	6 (27.3%)	0	6 (27.3%)	0
Diarrhoea	8 (36.4%)	1 (4.5%)	5 (22.7%)	1 (4.5%)
Fatigue	7 (31.8%)	1 (4.5%)	5 (22.7%)	0
Neutropenia or ANC decreased	7 (31.8%)	6 (27.3%)	5 (22.7%)	5 (22.7%)
Aspartate aminotransferase increased	5 (22.7%)	0	3 (13.6%)	0
Headache	5 (22.7%)	1 (4.5%)	3 (13.6%)	1 (4.5%)
Platelet count decreased	4 (18.2%)	3 (13.6%)	2 (9.1%)	1 (4.5%)
Insomnia	3 (13.6%)	0	2 (9.1%)	0
Anaemia	7 (31.8%)	5 (22.7%)	1 (4.5%)	1 (4.5%)
Dyspnoea	4 (18.2%)	1 (4.5%)	1 (4.5%)	0
Hypokalaemia	4 (18.2%)	1 (4.5%)	1 (4.5%)	0
Muscular weakness	3 (13.6%)	0	1 (4.5%)	0
Abdominal pain	4 (18.2%)	0	0	0
Cough	4 (18.2%)	0	0	0
Pleural effusion	3 (13.6%)	0	0	0
Thrombocytopenia	3 (13.6%)	1 (4.5%)	0	0

*No Related TEAEs = Grade 5 as of Apr 22, 2021; ** ≥10% of patients

All Events	Cohorts 1 to 5 (N=22)*
Any Treatment Emergent Adverse Events (TEAEs)	20 (90.9%)
Any TEAEs ≥ Grade 3	15 (68.2%)
TEAE Leading to Treatment Discontinuation	4 (18.2%)
TEAE Leading to Death	0 (0.0%)
Any Serious TEAEs	8 (36.4%)
Any Luxeptinib Related TEAEs ≥ Grade 3	9 (40.9%)‡
Any Luxeptinib Related Serious TEAEs	3 (13.6%)†
Dose Limiting Toxicity	1 (4.5%)††

* Data-cut date: Apr 22, 2021

‡ Including 2 patients who experienced Grade 3 lymphocytosis

† All three were assessed as possibly related to study.

†† One patient (Dose level 5, 750mg) had new onset hypertension during screening (Grade 1) and on C1D1 (Grade 2), which became Grade 3 on C1D6 and then Grade 4 hypertension and were assessed as **possibly** related to study drug.

- Safety data cut verification as of **April 22, 2021**
- Additional preliminary patient data as of **June 7, 2021**
  - 23 patients treated across 5 cohorts & 6 patients on study
- Patients heavily pre-treated with as many as 12 prior therapies
- Currently dosing Cohort 5 with 750mg BID
- **No safety trends to date that could prevent further dose escalation**

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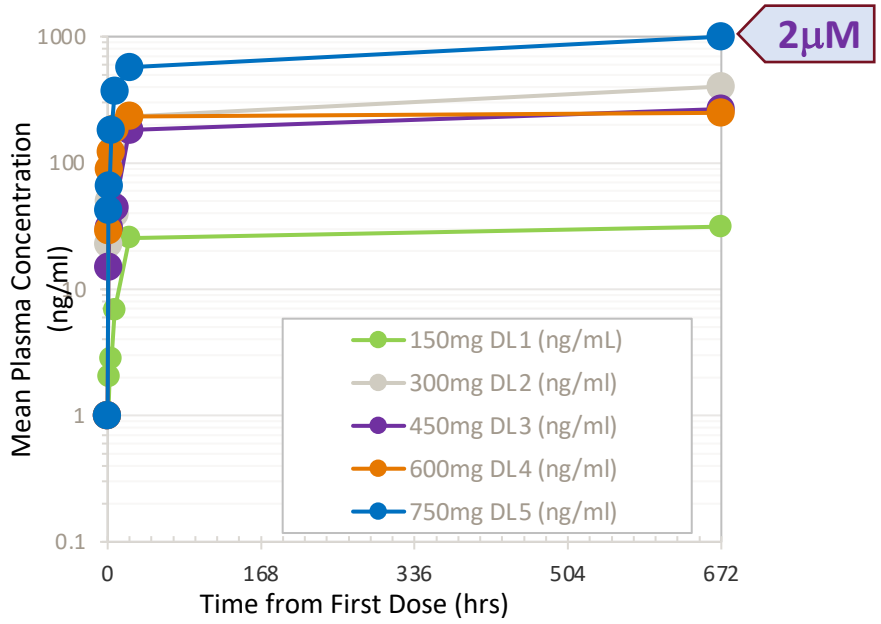
Dose Dependent Increases in Steady State *(trough)* PK in Patients with Relapsed or Refractory B-Cell Malignancies Treated with Luxeptinib

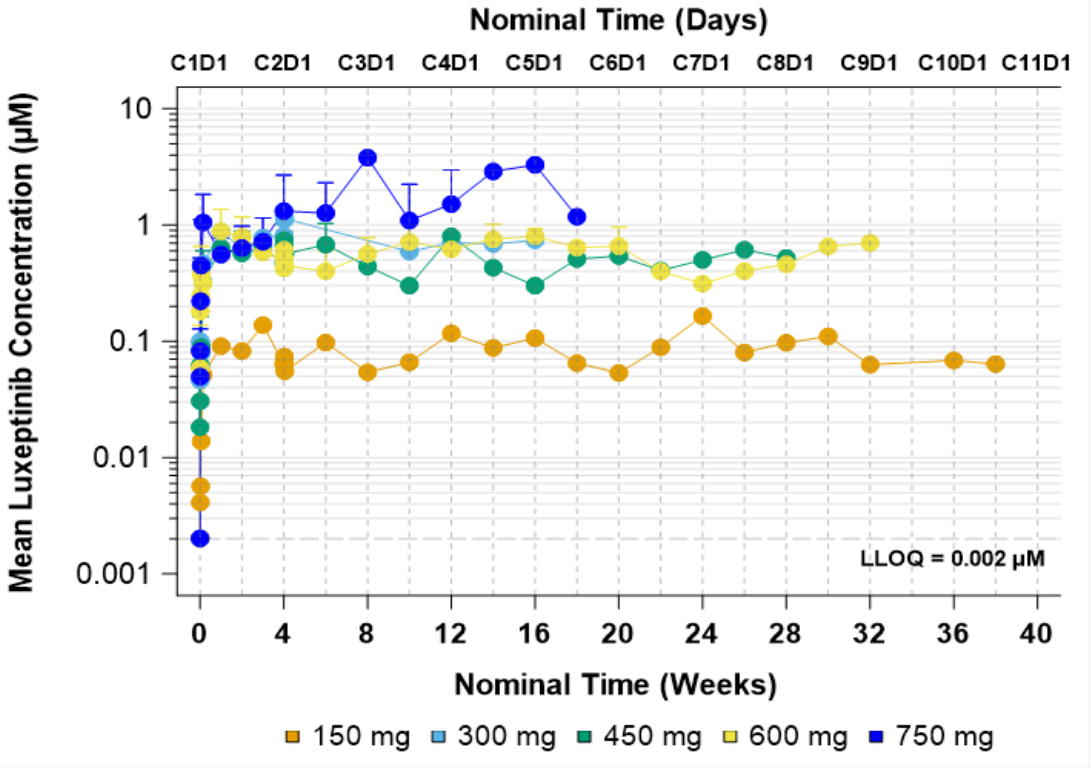
Mean Plasma PK Profile During Cycle 1 (28 days)



Luxeptinib achieved dose related pharmacokinetics and a steady state plasma concentration >2µM at the end of Cycle 1 (28 days) at the dose of 750mg BID.

Plasma PK Profile Over Multiple Cycles

Luxeptinib achieved dose-related steady state plasma concentrations



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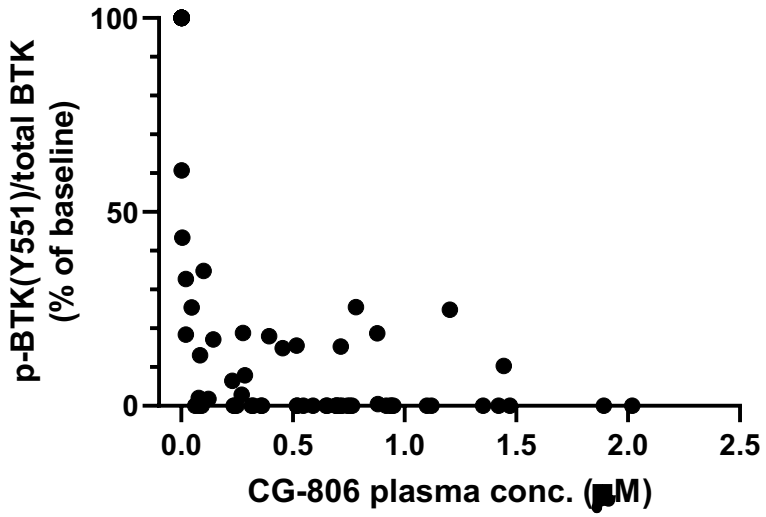
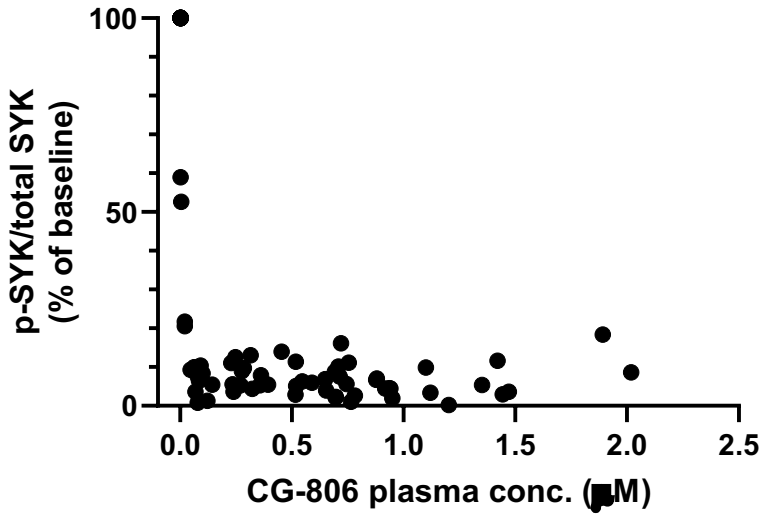
Higher Luxeptinib Exposures Lead to More Consistent PD Inhibition of p-BTK and p-SYK in Patients with Relapsed or Refractory B-Cell Malignancies

BTK-pY551 Inhibition



SYK-pY525/526 Inhibition



Study Cohorts 1–5 (n=13): Target Engagement by Plasma Inhibitory Activity (PIA) Assay [*PIA Assay Provides a Surrogate for In Vivo Target Inhibition*]

EOL-1 cells were used as a reporter cell line, since they express many of the kinases targeted by luxeptinib (CG-806). Cells were treated for 6 hours with plasma collected from patients at the indicated timepoints and then subjected to whole cell lysis and immunoblotting. Kinase activity as a function of dose was determined via densitometry analysis.

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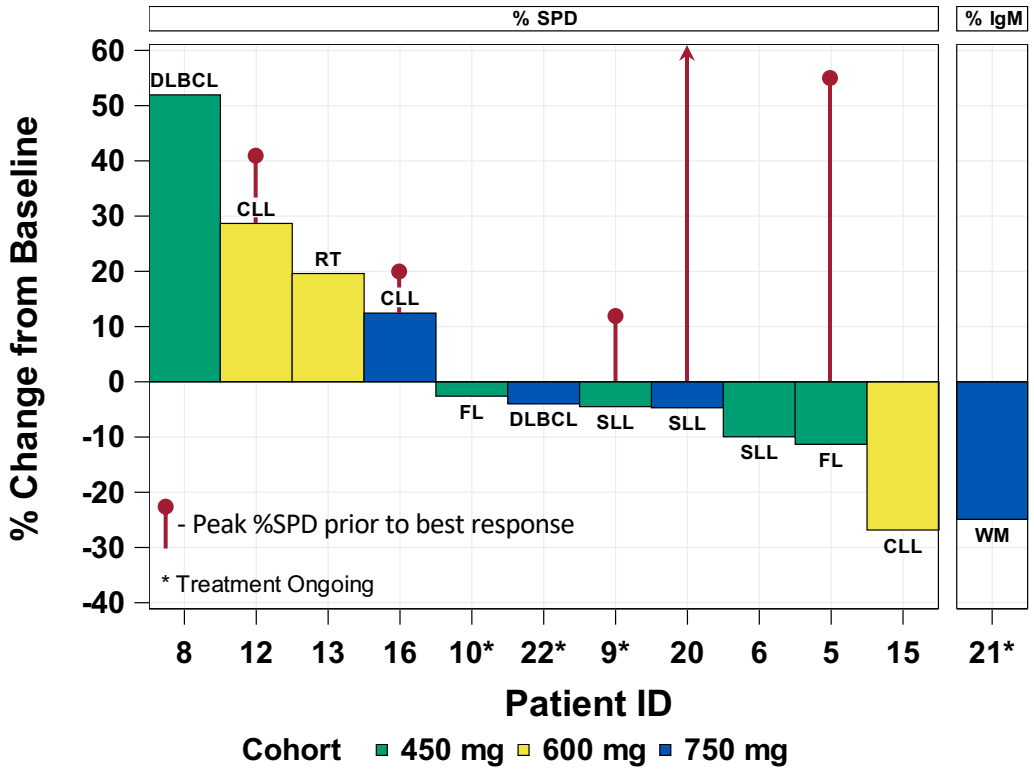
Waterfall Plot of Best Response in Patients with Relapsed or Refractory B-Cell Malignancies Treated with Luxeptinib

Best Response in Evaluable Patients Treated in Various Cohorts

All patients, who had at least one imaging for tumor measurements or IgM measurement (WM patient) since starting treatment, were included (n=12). Includes preliminary data through **June 7, 2021**



Heavily-pretreated B-cell cancer patients

- Range of 2-12 prior regimens
- Many patients rapidly progressed immediately before Lux treatment was initiated

Observing trend of tumor growth early in treatment, often followed by tumor reductions

Emergence of dose-dependent anti-leukemic activity to Lux in patients who received dose escalation

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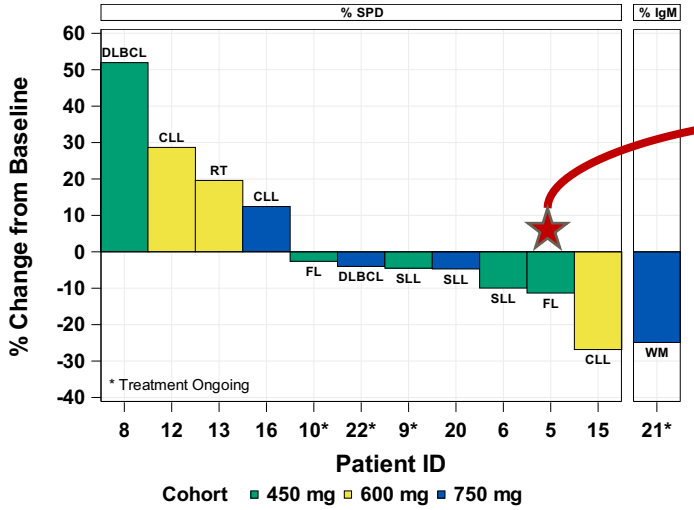
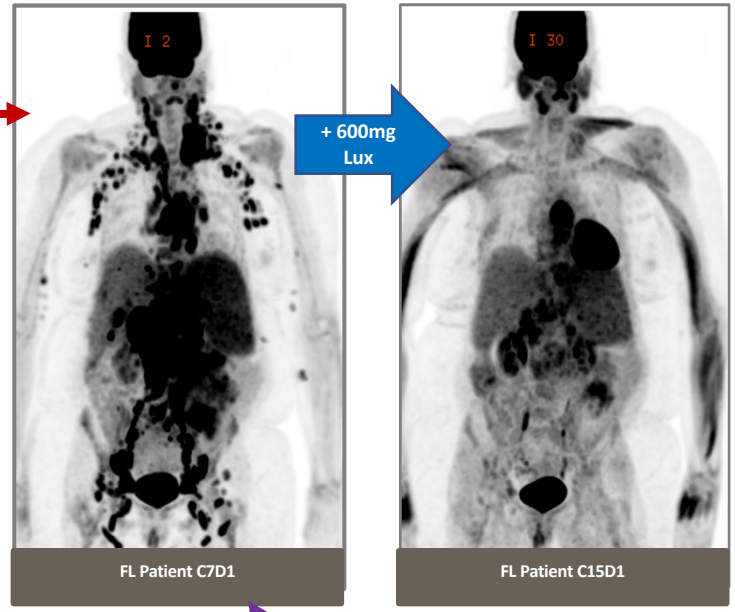
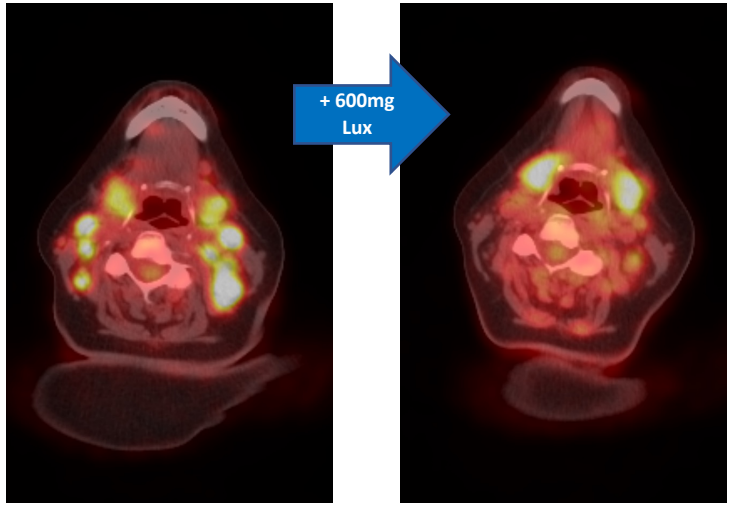
Luxeptinib Anti-leukemic Activity in Follicular Lymphoma Patient: Case Study Patient #5 (450mg BID and 600mg BID)

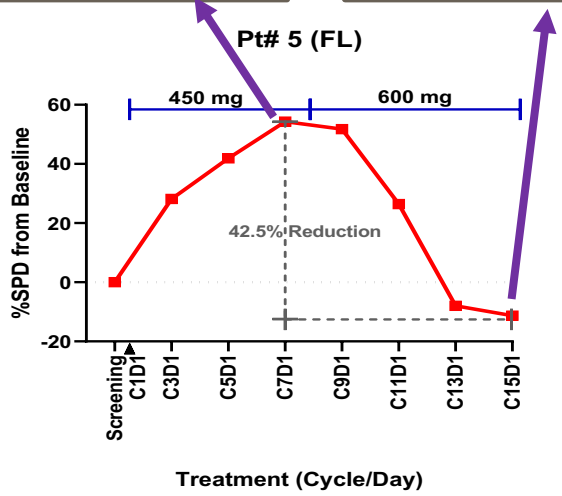


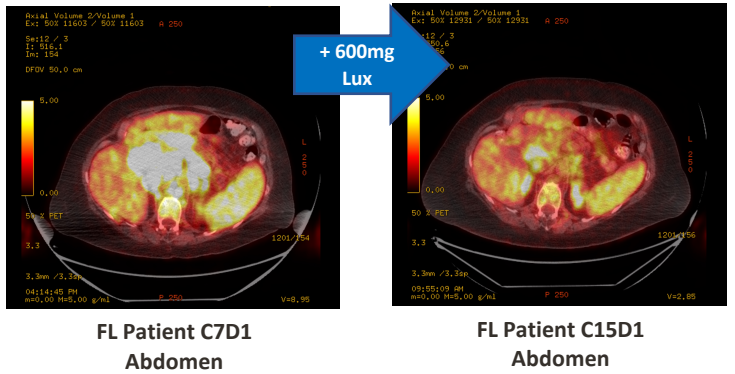




Patient #5 (FL) exemplifies how waterfall underrepresents anti-leukemic effect

- Tumor growth while on 450mg BID
- Upon dose escalation to 600mg BID, patient experienced 43% tumor reduction from peak (12% from baseline)
- Well-tolerated with single agent activity for the duration of 16+ cycles of therapy





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Luxeptinib Phase 1a/b in B-cell Tumors: Findings To Date and Next Steps

Intermediate dose levels to date have delivered leading indicators of clinical activity

- **Well tolerated** across five dose levels and multiple disease types
- Target engagement with **dose-dependent inhibition of phospho-BTK**
- **Treatment-related lymphocytosis** in patients presenting with classic CLL
- **Tumor reductions** across **different B-cell malignancies** (FL, CLL, SLL, WM)
- **Intra-patient dose-dependent antitumor activity** warrants continued dose escalation

Continuing to higher doses and longer exposures to tackle an increasingly challenging population

- R/R CLL and NHL patients now are **more clinically challenging than in prior comparable studies**
- **Higher drug dose and longer exposure** may affect this heavily pretreated population
- Currently treating patients at 750mg, and **plan to dose escalate further**
- Plan to continue exploring **multiple lymphomas**, in line with anti-tumor activity to date

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Luxeptinib (CG-806)

Phase 1a/b Trial for Patients with Acute Myeloid Leukemia (AML)

Luxeptinib Progressing in Phase 1a/b Clinical Trial in R/R AML Patients

- Broadly potent against AML cells, suggesting potential across entire AML patient population
- Initiated dosing with 450mg BID as a potentially active dose; now escalated to 750mg BID in Cohort 3
- Observed anti-leukemic activity, including a patient with a complete response (CR)
- Potential to rapidly differentiate from approved FLT3 inhibitors

Cohort	Dose	Status
1	450 mg BID	Completed
2	600 mg BID	Completed
3	750 mg BID	Ongoing
4	900 mg BID	Planned

Include R/R AML Patients with Unmet Needs

- Patients who failed FLT3 inhibitors
- Patients who failed IDH inhibitors
- Patients who failed venetoclax
- Patients with mutated p53, mutated RAS
- Patients with wild type-FLT3
- Patients unfit for intensive therapies
- Patients who failed bone marrow transplants

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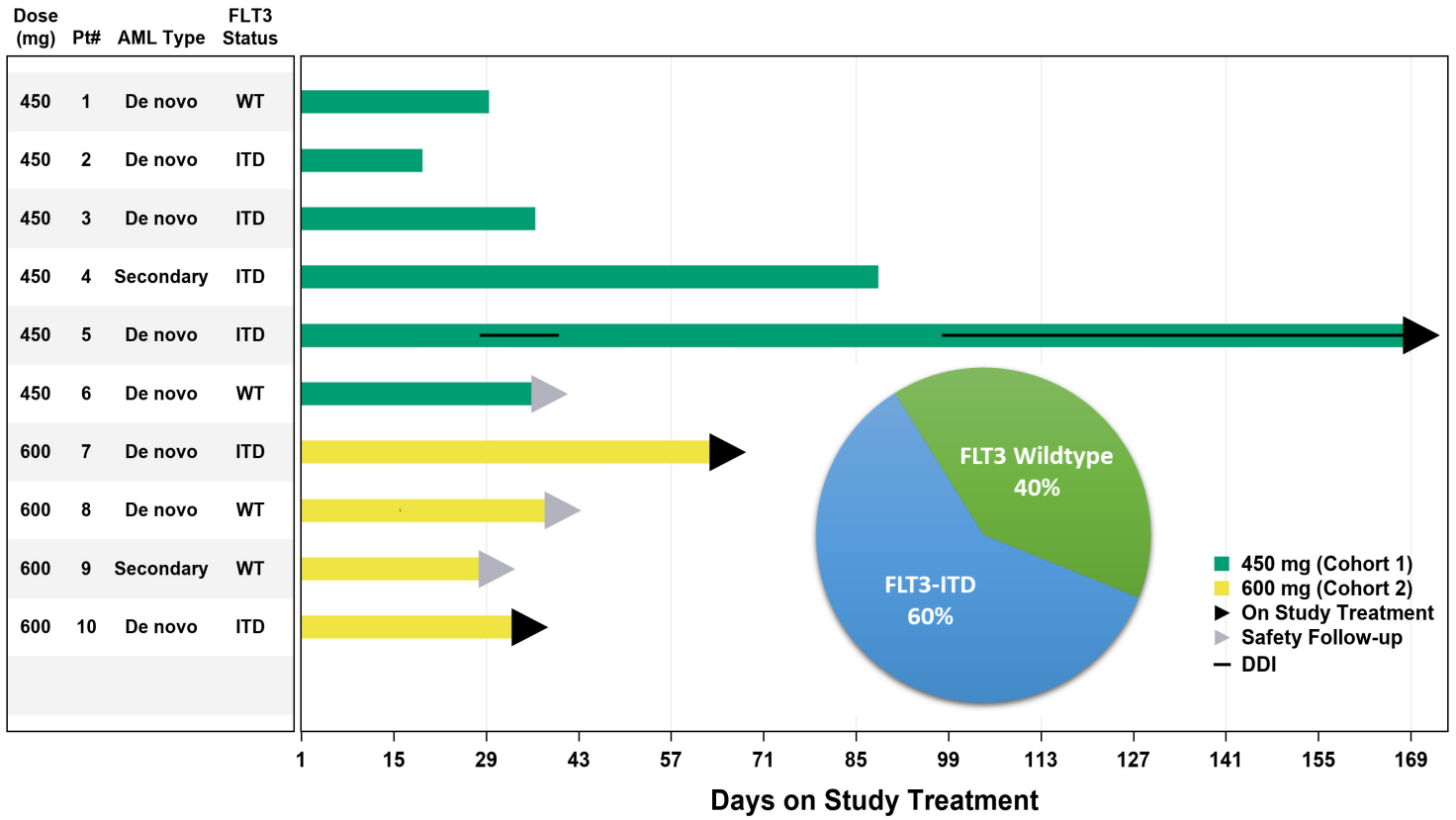
Swimmers' Plot and Demographics of Patients with Relapsed or Refractory AML Treated with Luxeptinib (Data Cut: April 22, 2021)



Patient Demographics	Cohorts 1 to 2 (N=9)*
Median Age (Range), Years	**74.0 (36, 81)**
Sex, N (%)	
Male	**7 (77.8%)**
Female	**2 (22.2%)**
Ethnicity, N (%)	
Not Hispanic or Latino	**6 (66.7%)**
Hispanic or Latino	**0 (0.0%)**
Not Reported	**3 (33.3%)**
Race, N (%)	
White	**7 (77.8%)**
Asian	**1 (11.1%)**
Other	**1 (11.1%)**
ECOG Score, N (%)	
0 -Normal activity	**2 (22.2%)**
1 -Symptoms, but ambulatory	**7 (77.8%)**
FLT3 Mutation Status, N (%)	
WT	**4 (44.4%)**
ITD	**5 (55.6%)**
AML Type, N (%)	
De novo	**7 (77.8%)**
Secondary AML	**2 (22.2%)**
Relapsed or Refractory, N (%)	
Relapsed	**1 (11.1%)**
Refractory	**3 (33.3%)**
Both Relapsed and Refractory	**5 (55.6%)**
RBC Transfusion Dependent, N (%)	**6 (66.7%)**
Platelet Transfusion Dependent, N (%)	**5 (55.6%)**
Median Number of Lines of Prior Therapy (Range)	**3 (1, 8)**
Chemotherapy, N(%)	**4 (44.4%)**
Radiation	**1 (11.1%)**
Targeted and Immunotherapy, N (%)	
Hypomethylating Agent **	**9 (100%)**
Anti-BCL2 (venetoclax)	**8 (88.9%)**
Kinase Inhibitor†	**5 (55.6%)**
Allogeneic stem cell transplantation	**3 (33.3%)**
IDH1-Inhibitor (ivosidenib)	**1 (11.1%)**
Immunotherapy††	**1 (11.1%)**
Other Experimental Agent	**1 (11.1%)**

*Data-cut date: Apr 22, 2021

**Four patients were on azacitidine, three patients on decitabine, and two patients on both

†Including sorafenib, ruxolitinib, crenolanib, or gilteritinib; ††Including ipilimumab.

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Safety and Tolerability Profile of Patients with Relapsed or Refractory AML Treated with Luxeptinib (Data Cut: April 22, 2021)

Events	Cohorts 1 to 2 (N=9)*
Any Treatment Emergent Adverse Events (TEAEs)	8 (88.9%)
Any TEAEs ≥ Grade 3	6 (66.7%)
Any CG-806 Related TEAEs ≥ Grade 3	3 (33.3%)
TEAE Leading to Treatment Discontinuation	1 (11.1%)
TEAE Leading to Death	1 (11.1%)
Any Serious TEAEs	4 (44.4%)
Any CG-806 Related Serious TEAEs	1 (11.1%)†
Dose Limiting Toxicity	1 (11.1%)††

* Data-cut date: Apr 22, 2021

† One patient in Cohort 1 (450mg, BID) had Grade 3 pericardial effusion and Grade 2 pleural effusion, both assessed as possibly related to study drug.

†† One patient had Grade 3 pericardial effusion, as stated above in note †.

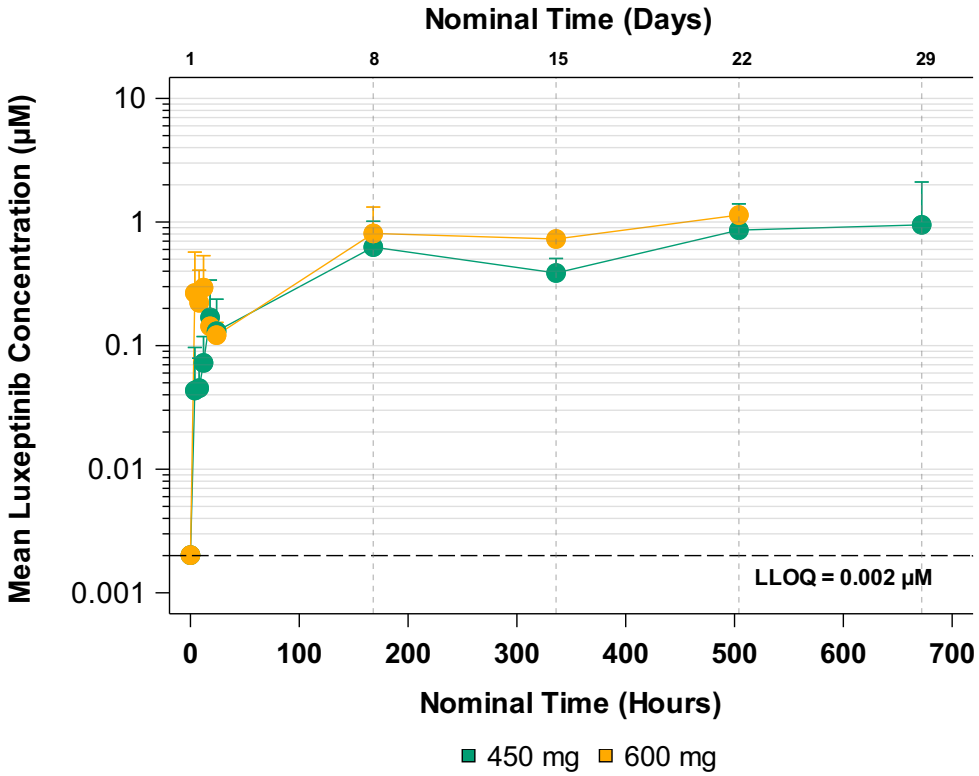
Luxeptinib Related Treatment Emergent Adverse Events			
Preferred Term	Cohorts 1 to 2 (N=9)*		
	Any Grade, N (%)	Grade 3, N (%)	Grade 4, N (%)
Nausea	3 (33.3%)	0	0
Fatigue	2 (22.2%)	0	0
Platelet count decreased	2 (22.2%)	0	2 (22.2%)
Activated partial thromboplastin time prolonged	1 (11.1%)	0	0
Anaemia	1 (11.1%)	1 (11.1%)	0
Blood alkaline phosphatase increased	1 (11.1%)	0	0
Decreased appetite	1 (11.1%)	0	0
Headache	1 (11.1%)	0	0
Hyperphosphataemia	1 (11.1%)	0	0
Insomnia	1 (11.1%)	0	0
Neutropenia or ANC decreased	1 (11.1%)	1 (11.1%)	0
Pericardial effusion	1 (11.1%)	1 (11.1%)	0
Photophobia	1 (11.1%)	0	0
Pleural effusion	1 (11.1%)	0	0

*No luxeptinib related TEAEs = Grade 5 as of Apr 22, 2021

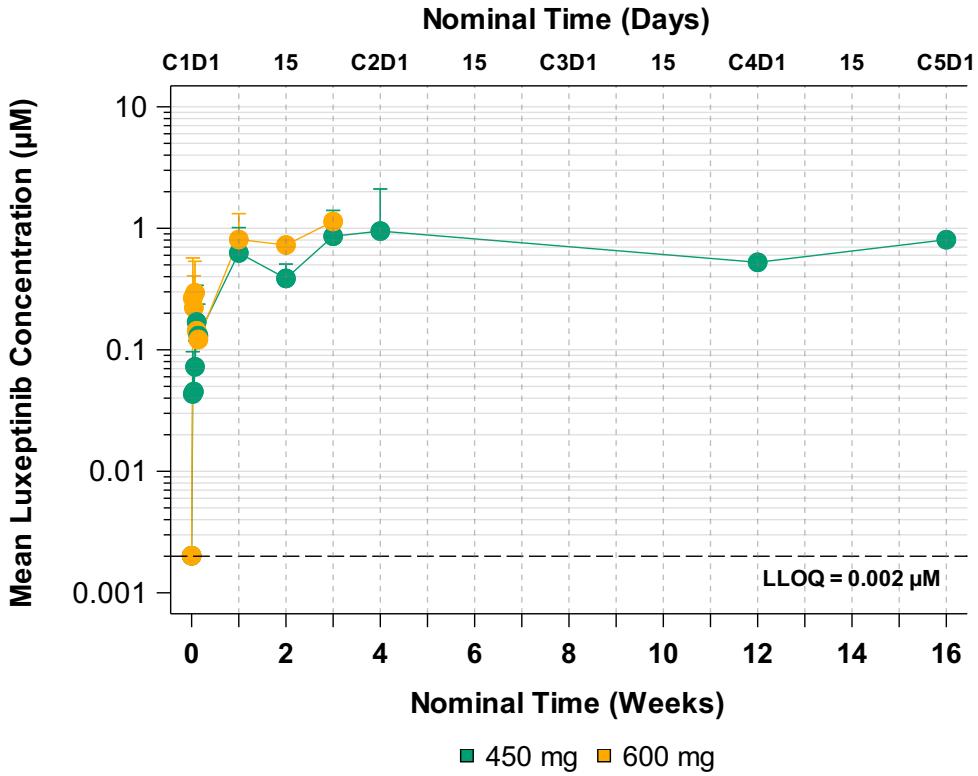
- **Cohort 1 (450mg BID): Patient #2 entered the trial with a history of gilteritinib-associated myopericarditis and tapering of corticosteroids**
  - Developed Gr 3 pericardial effusion midway through Cycle 1 while taking 450mg BID Lux, but possibly associated with pre-existing observations
  - Findings also consistent with potential differentiation syndrome, but patient withdrew from the study before determinations could be made. Accordingly, the protocol required this be assessed as a DLT possibly related to study drug and led to the expansion of Cohort 1 to 6 patients.
  - No DLT in 5 other patients in Cohort 1, CSRC assessed a protocol-mandated DLT and went on to approve dose escalation to Cohort 2.
- **Cohort 2 (600mg BID): No DLT or other safety concerns reported in 4 patients, supporting dose escalation to Cohort 3**
- **Cohort 3 (750mg BID): Ongoing**

Steady State *(trough)* PK in Patients with Relapsed or Refractory AML Treated with Luxeptinib

Mean Plasma PK Profile During Cycle 1 (28 days)



Plasma PK Profile Over Multiple Cycles



Luxeptinib achieved steady-state plasma concentrations of approximately 1uM for 600mg BID treatment, consistent with the PK profile from the clinical trial in B-cell malignancies.

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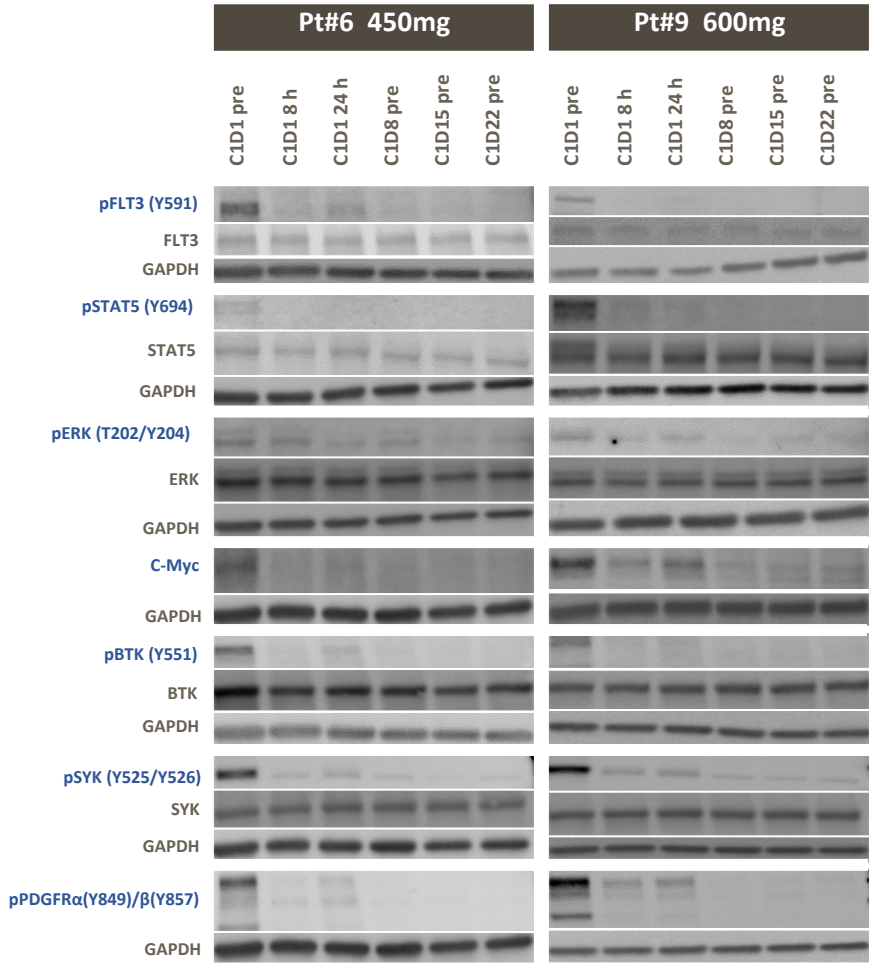
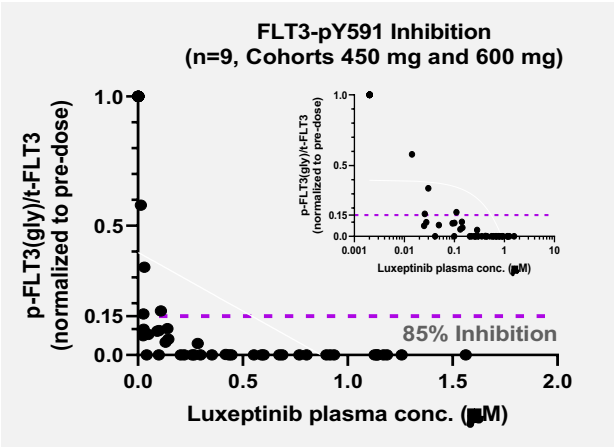
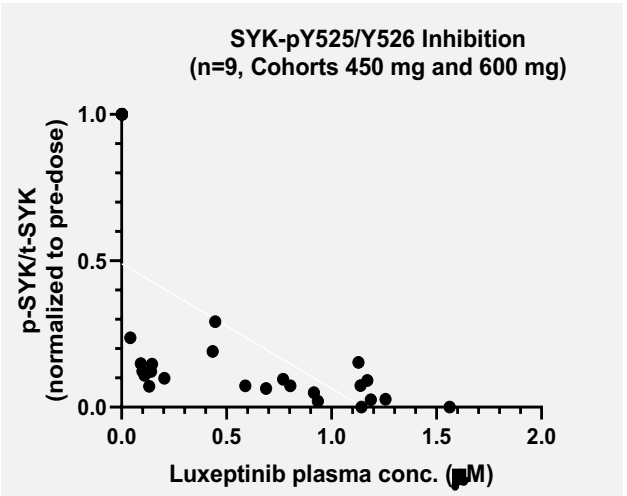
PD Activity in AML Patients Receiving 450mg and 600mg Luxeptinib: Dose Dependent Inhibition of Signaling of FLT3, SYK, BTK, and PDGFRα







Target Engagement by Luxeptinib in PIA assay (n=9, Cohorts 1 and 2):

Surrogate for In Vivo FLT3 Inhibition

- Steady state plasma levels deliver 100% inhibition of FLT3-pY591
- Dose-dependent inhibition of FLT3 downstream signaling (pFLT3, pSTAT5, pERK and c-MYC)
- Dose-dependent inhibition of non-FLT3 survival pathways (pSYK, pBTK and pPDGFα)

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Luxeptinib Anti-leukemic Activity in AML Patient: Case Study Patient #3 in First Cohort (450mg BID)

Heavily-pretreated FLT3-ITD Tx-relapsed *de novo* AML

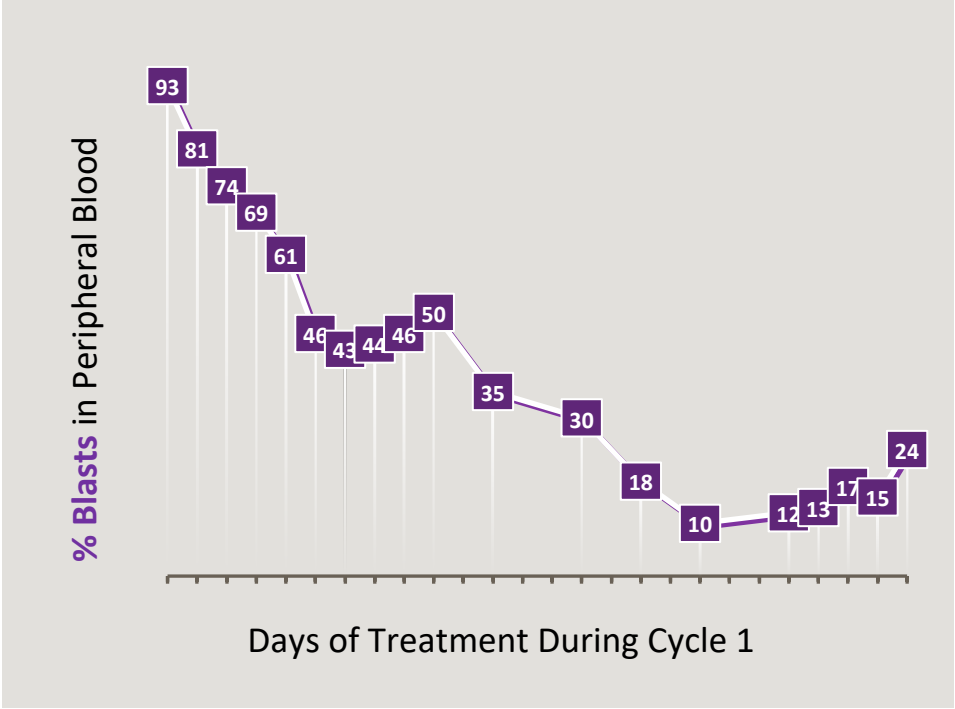
36 y.o., Female

- Eight (8) prior therapies: chemotherapy, azacitidine, venetoclax, allogeneic transplant, **gilteritinib FLT3i**, **crenolanib FLT3i**
- Mutations detected at screening: FLT3-ITD, DNMT3A, NPM1, GATA2, WT1
- Aggressively progressed before Lux treatment : Blasts increased from $0.33x10^3/\mu L$ in peripheral blood at screening (-12 days) to $6.38x10^3/\mu L$ on C1D1.

Luxeptinib 450mg BID

- 90+% reduction of blasts in cycle 1, before disease progression in C2
- Blasts in peripheral blood were reduced from $6.38x10^3/\mu L$ on C1D1 to $0.79x10^3/\mu L$ on C1D8 (↓88%) and $0.09x10^3/\mu L$ on C1D15 (↓ 99%)
- FLT3-ITD VAF: 0.77 in BM at screening and 0.63 in peripheral blood on C2D1

Lux at 450mg BID targeted the FLT3-ITD but an aggressive clone persisted



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Luxeptinib Anti-leukemic Activity in AML Patient: Case Study Patient #4 in First Cohort (450mg BID)

FLT3-ITD Tx-relapsed *de novo* AML

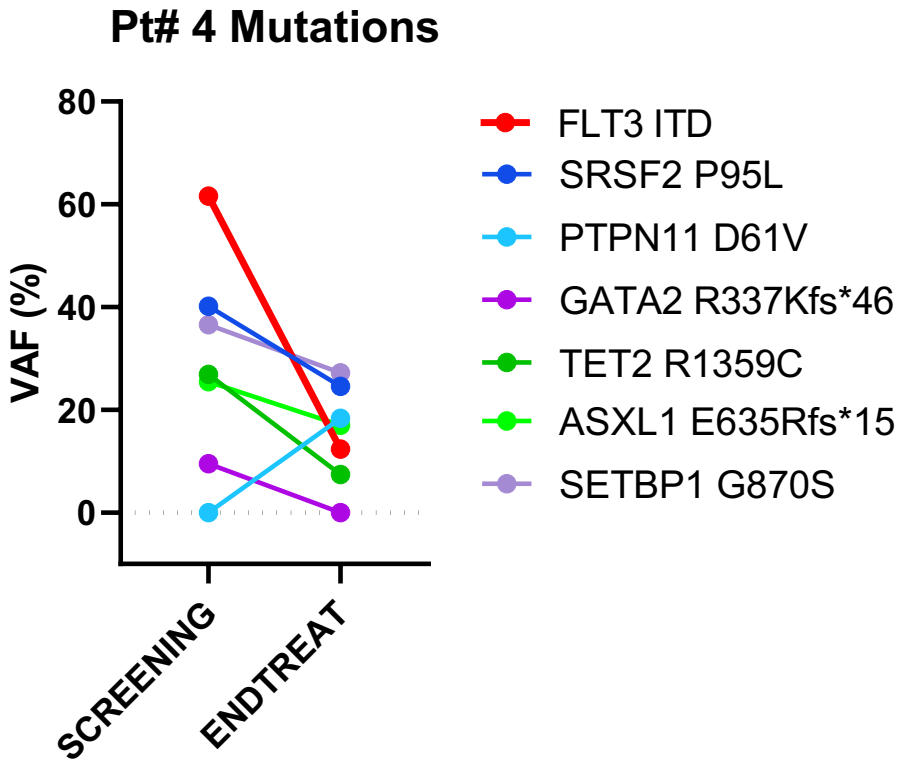
76 y.o., Male

- Two (2) prior therapies: azacitidine, venetoclax
- Mutations detected at screening: *FLT3*-ITD, *TET2*, *ASXL1*, *SRSF2*, *SETBP1*, *GATA2*
- Blast increase before Lux treatment : Blast in peripheral blood increased from 4% on the day before dosing to 9% on C1D1

Luxeptinib 450mg BID

- Blasts in peripheral blood continuously decreased to 3% by the End of Tx
- **FLT3-ITD VAF (↓ 80%)** from 0.62 in peripheral blood at screening to 0.12 at the end of treatment (C4D14)
- Lux **reduced VAF** of ***GATA2* R337K (↓ 100%)**, ***TET2* R1359C (↓ 73%)**, ***SRSF2* P95L (↓ 39%)** and ***ASXL1* E635R (↓ 33%)** mutants associated with poor outcomes
- PTPN11 mutation was detected with VAF 18% at the end of treatment

Lux at 450mg BID effectively targeted the FLT3-ITD mutated clone over multiple cycles



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Luxeptinib Delivers MRD-negative Complete Response in AML Patient
Case Study Patient #5 in First Cohort (450mg BID)

FLT3-ITD Tx-relapsed *de novo* AML / myeloid sarcoma (extra medullary perispinal mass)

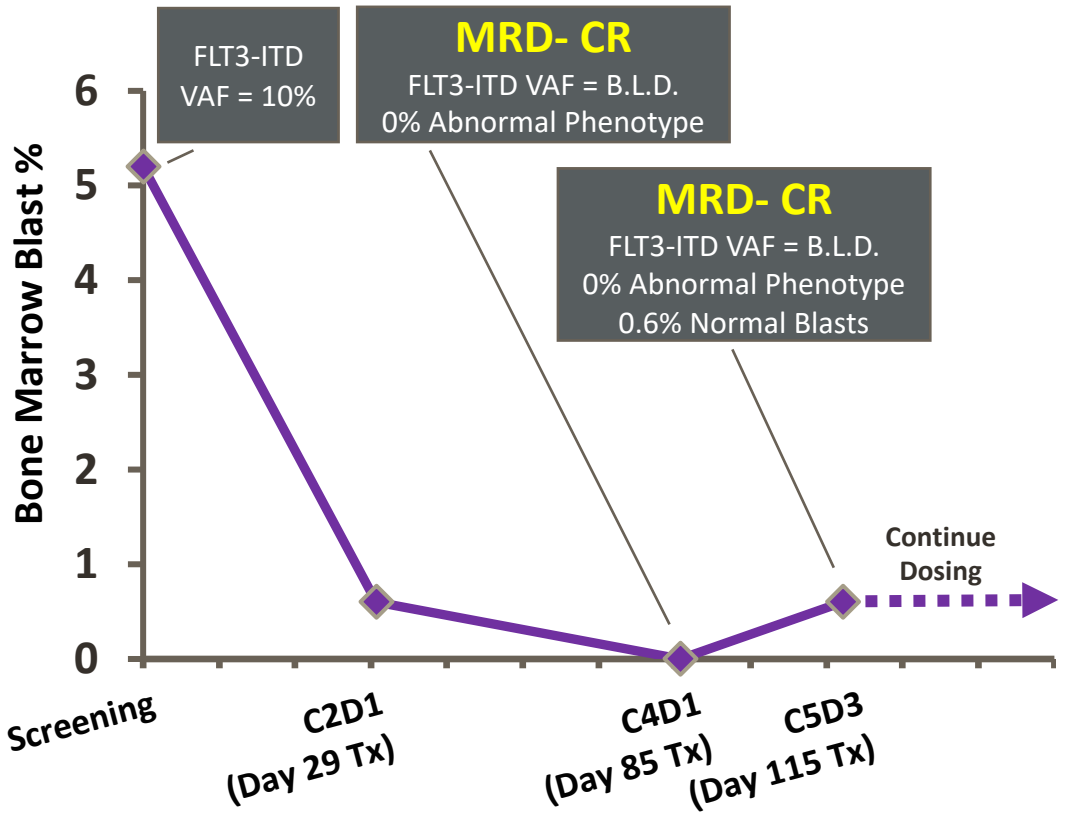
46 y.o., Male

- Induction chemotherapy
- Salvage chemotherapy + **sorafenib FLT3i** followed by AHSC Transplant #1
- Relapsed 2.5 years later, treated with:
  Decitabine + venetoclax + **sorafenib FLT3i** followed by AHSC Transplant #2
- Extramedullary relapse near spine 8 months later & increased BM blasts:
  Received focal radiation to perispinal mass just prior to screening

Luxeptinib 450mg BID

- Bone marrow aspirate blast reduced from 5.2% at screening to 0.6% on C2D1 and remained <1% thereafter, without myelosuppression
- Bone marrow *FLT3*-ITD VAF below detection limit at C2D1, C4D1, & C5D3
- Highly sensitive flow cytometry failed to detect abnormal blasts in bone marrow at C4D1 and C5D3 (<0.1%)

✓ MRD-negative Complete Response by HS-flow cytometry
✓ Patient Continues on Study in Cycle 7



Hgb (g/dL):	10.0	12.7	11.3	11.6
Plat (K/ml):	174	189	163	229
ANC (K/ml):	1.31	1.30	1.98	1.55

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Luxeptinib Phase 1a/b in AML: Findings To Date and Next Steps

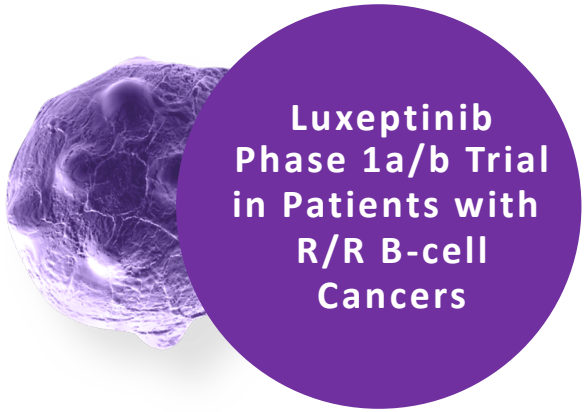
The first two dose cohorts delivered encouraging anti-leukemic activity

- **Durable MRD-negative CR in FLT3-ITD AML patient** who had failed 2 rounds of transplant and FLT3i
- **Meaningful anti-blast activity in FLT3-ITD AML patient** who had failed 8 prior therapies including FLT3i
- Achieved anticipated **steady state PK levels and PD inhibition of target kinases**, in line with prior studies
- **Completed 450mg and 600mg cohorts with no safety trends** likely to prevent continued dose escalation

Continuing dose escalation, and preparing strategy for multiple expansion cohorts

- Currently in the 750mg cohort, and **plan to dose escalate further**
- Expect to select **expansion dose level and expansion cohort strategy in 2H21**
- Aim to explore **different AML genotypes**, under monotherapy and combination therapy

Acknowledgements: We thank our PIs, Site Staff, Patients and Families



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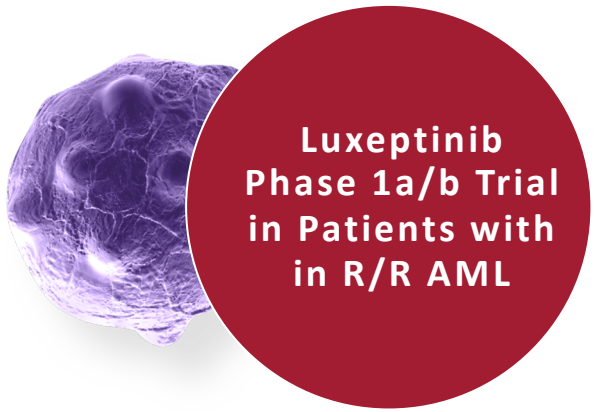

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