

# Galápagos

## SAPHIRA 1 topline results

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Webcast  
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# 2016: great execution year

- Closure of the deal with Gilead on filgotinib, \$725 M received in January
- Filgotinib: Ph 3 studies started in RA & CD, Ph 2/3 in UC
- Endoscopic data of filgotinib treatment in CD presented
- CF: all components of triple therapy in human trials
- CF collaboration with AbbVie expanded
- `1690 started Ph2a in IPF
- MOR106 antibody started in atopic dermatitis
- Galapagos included in BEL20, AEX and Stoxx Europe indices



# CF portfolio

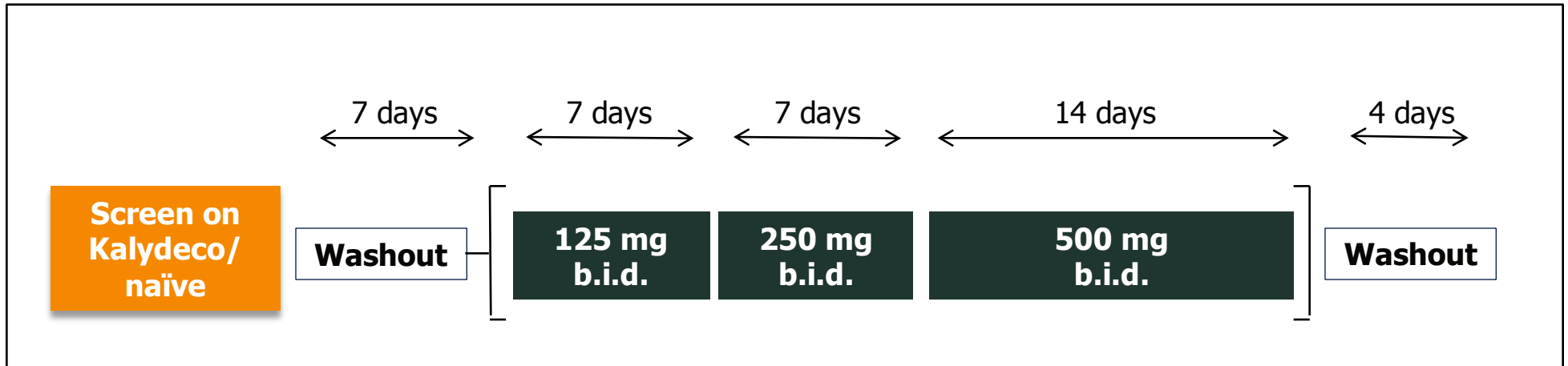
Preclinical	Ph1	Ph2	Status
potentiator '1837			Ph2 results: disclosed
potentiator '2451			Ph1 results: H1 '17
potentiator '3067			Ph1 start: H1 '17
C1 corrector '2222			Ph2 start: ✓
C1 corrector '2851			Ph1 start: H2 '17
C2 corrector '2737			Ph1 results: H1 '17
C2 corrector '3221			Ph1 start: H2 '17

On track to have triple in patients by mid-2017



# SAPHIRA 1

'1837 Ph2a open label trial

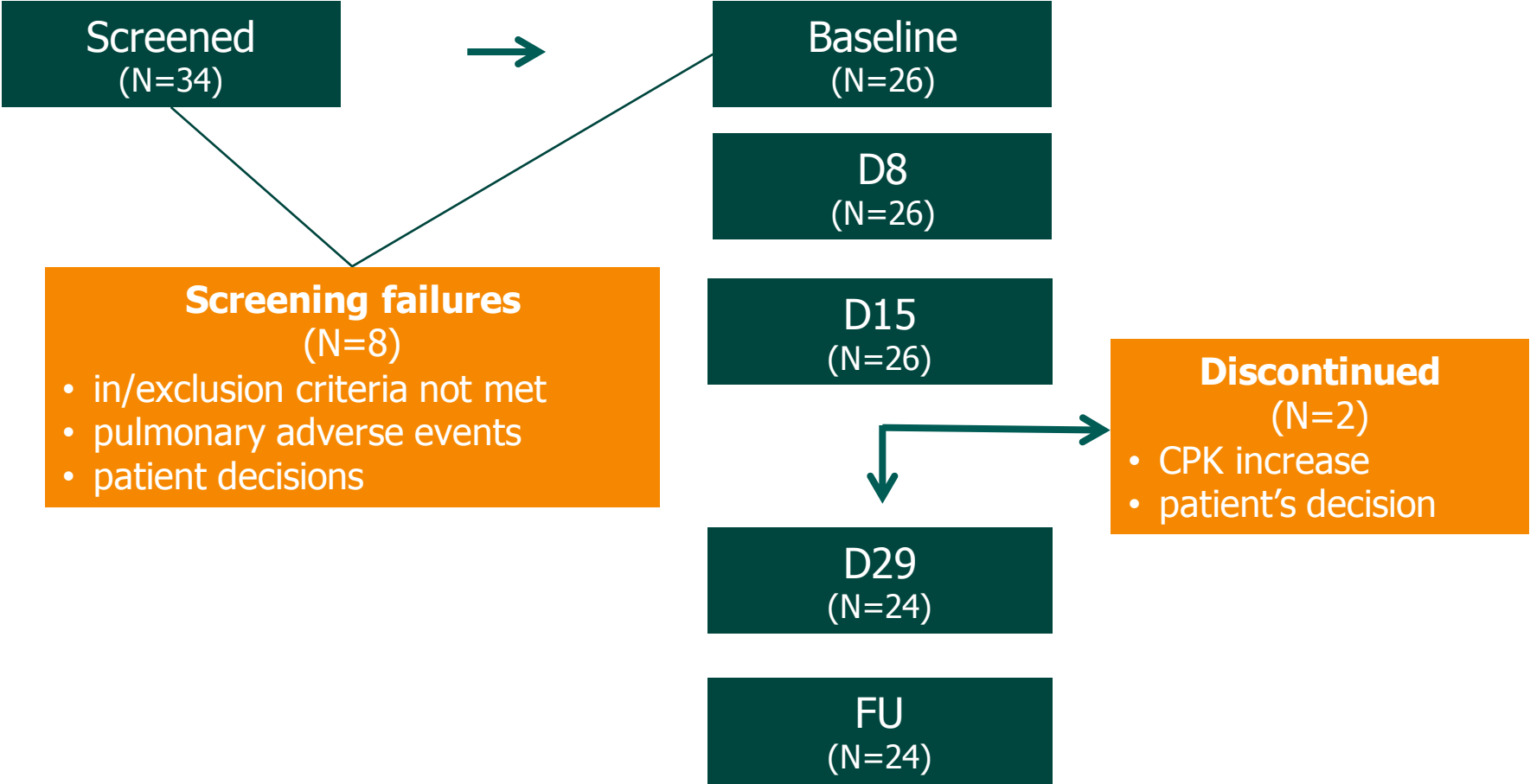


26 patients harboring a G551D mutation

- 25 Kalydeco treated & 1 naïve patient
- recruited at 16 centers in 6 EU countries & Australia
- study executed within 1 year
- primary endpoints: safety & tolerability
- secondary endpoints: sweat chloride, FEV1, plasma levels



# Patient disposition





# Baseline characteristics

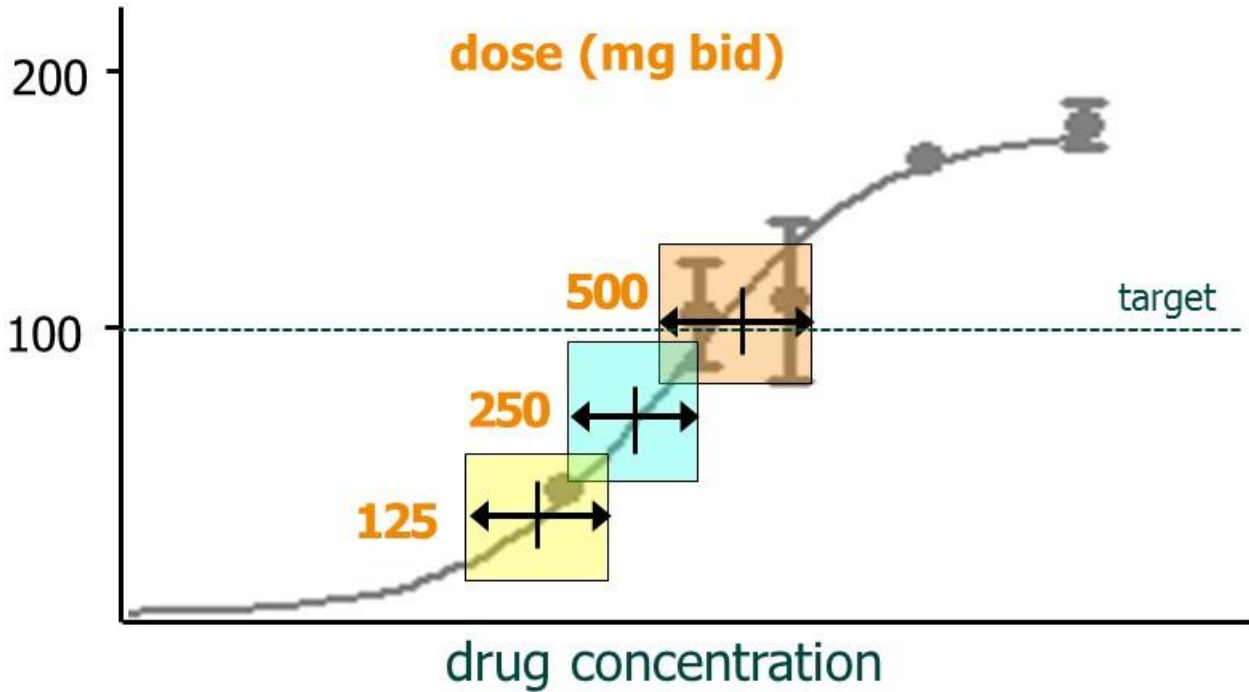
Age, years, mean	30
Range	19 - 51
Weight, kg, mean	68
Male, n (%)	12 (46%)
F508del on 2 <sup>nd</sup> allele	18/26 (69%)
[Sw Cl] <sub>@Baseline</sub> , mmol/L, mean (range)	98 (63 – 116)
Percent predicted FEV <sub>1</sub> , mean (range)	69 (30 – 104)
< 40%, n (%)	2 (8%)
40% – 60%, n (%)	7 (28%)
60% – 80%, n (%)	8 (32%)
> 80%, n (%)	8 (32%)
Kalydeco use, n (%)	25 (96%)
Mean duration, days (range)	1189 (310 – 2359)



# Dose selection

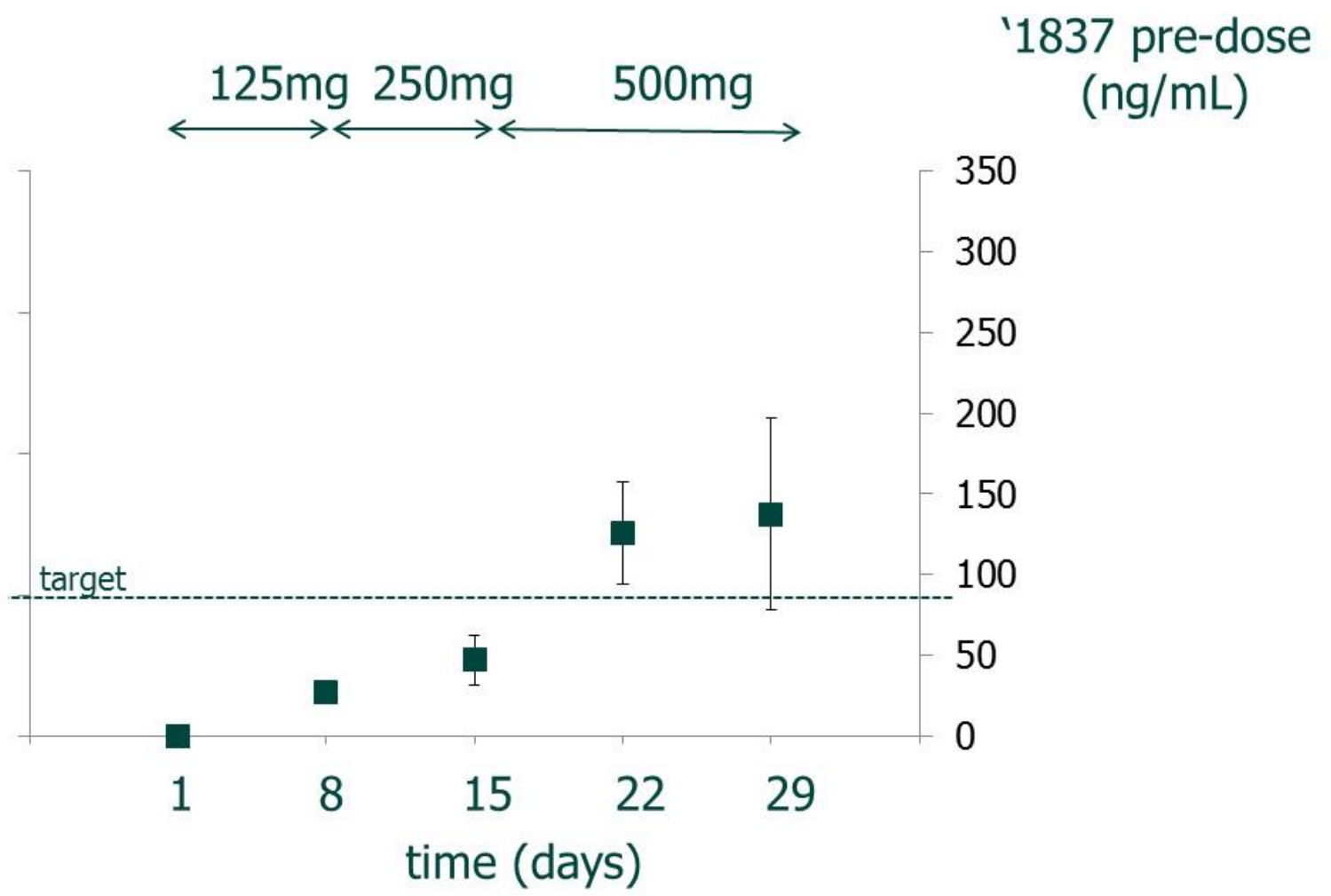
*In vitro* efficacy curve relative to predicted levels in G551D

% efficacy vs. Kalydeco

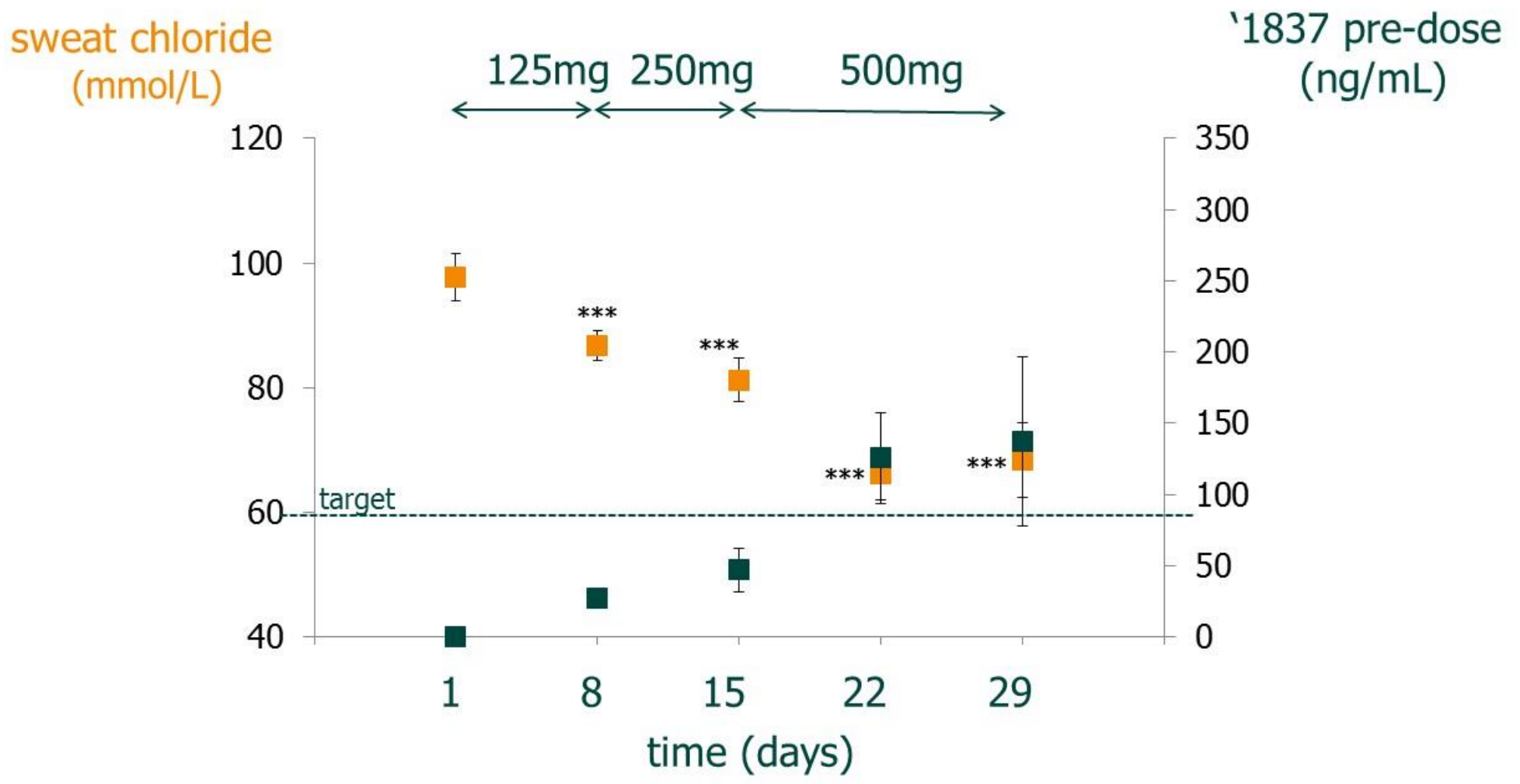




# Sweat chloride vs exposure



# Sweat chloride vs exposure





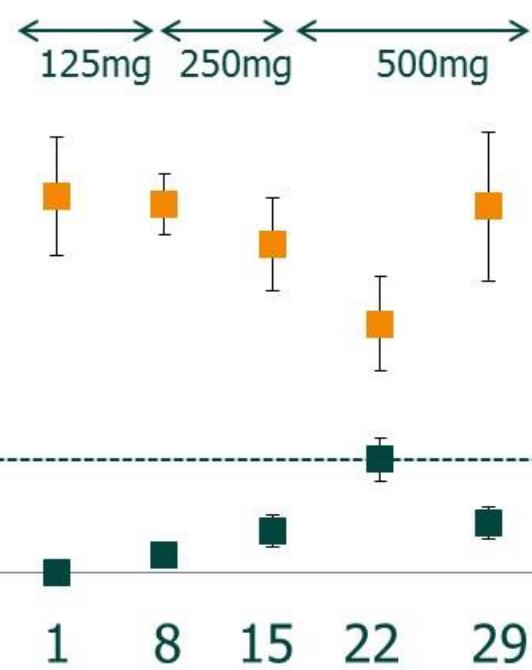
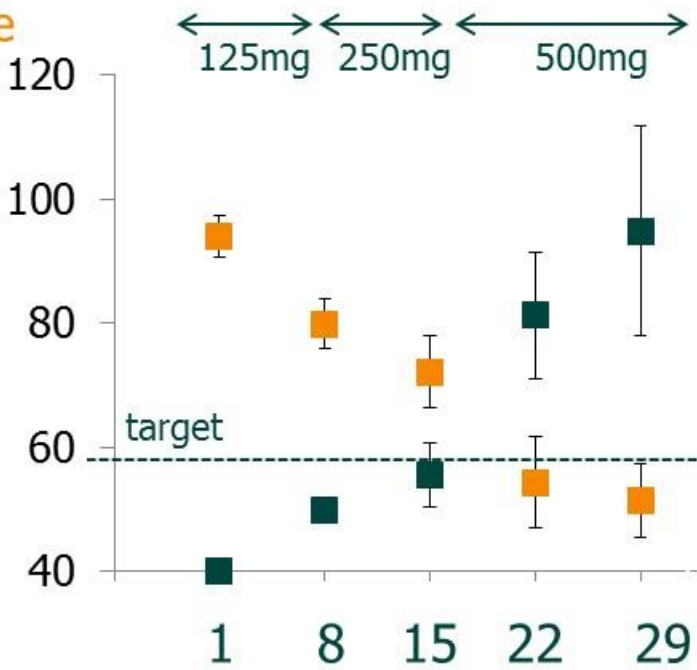
# Sweat chloride

'1837 exposure on Day 29

Exposure above target (N=15)

Exposure below target (N=6)

sweat chloride (mmol/L)

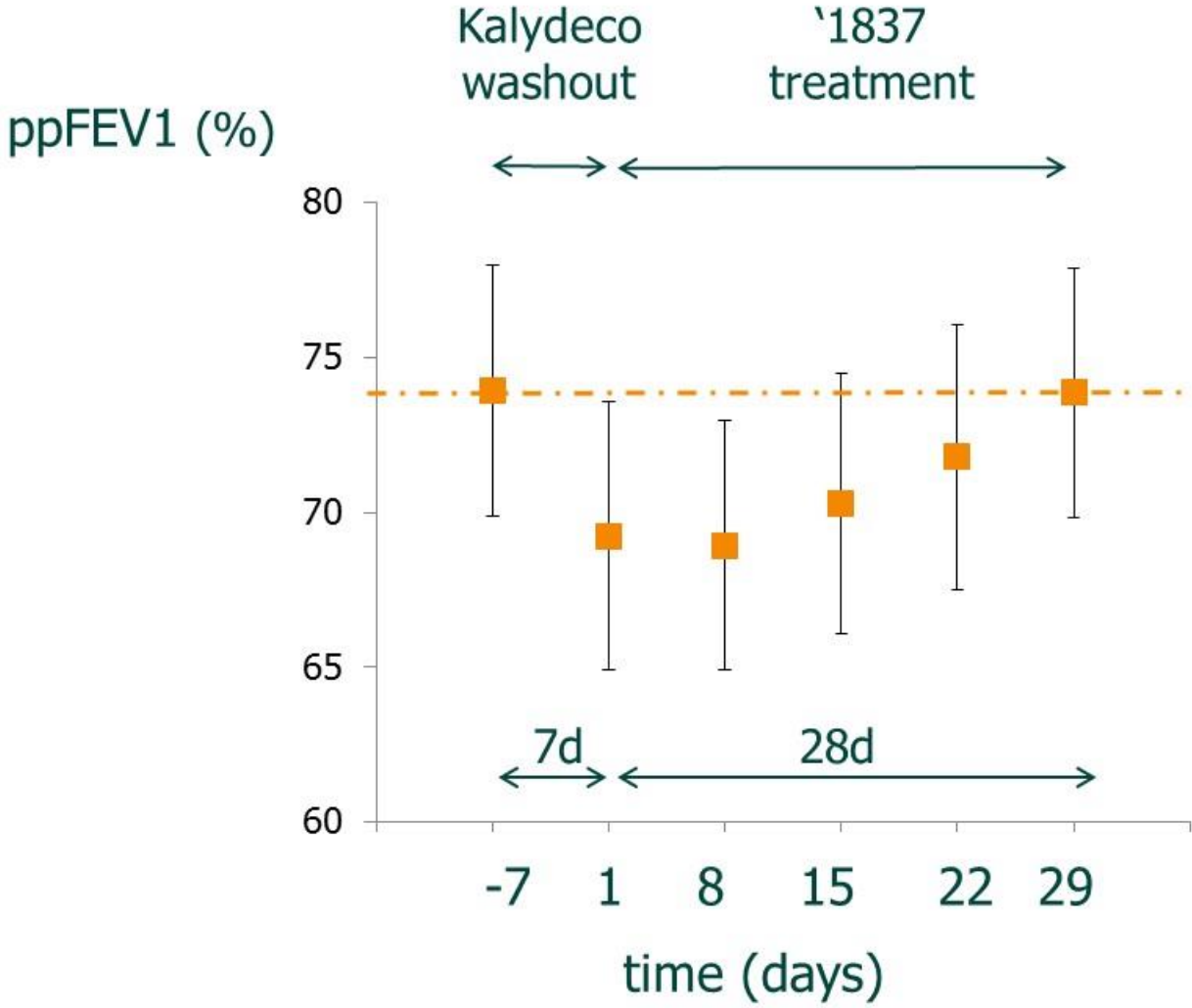


exposure (ng/mL)

time (days)



# Effect on FEV1





# Safety & tolerability

## Adverse events

- Generally well-tolerated
- Three serious adverse events in 2 patients
  - 1 patient with non-cardiac CPK increase
  - 1 patient with distal intestinal obstruction syndrome during screening period & pulmonary exacerbation (D28) of CF, resulting in hospitalization
- All other adverse events mild/moderate
- Most common adverse events: headaches, fatigue
- Some respiratory adverse events in 1<sup>st</sup> week
  - low incidence in weeks 2-4



# SAPHIRA 1 topline

## Conclusions on '1837

- First potentiator after Kalydeco to show positive results in G551D
- Appears safe & well tolerated
- Statistically significant decreases in sweat chloride
- Full restoration of FEV1 % loss from Kalydeco washout
- Supports our predictive *in vitro* assays
- Strengthening of our dosing modelling for triple combination