



**Interim Results from the
Open Label ACROBAT Edge Phase 2 Study and
Corporate Update**

SAFE HARBOR STATEMENT

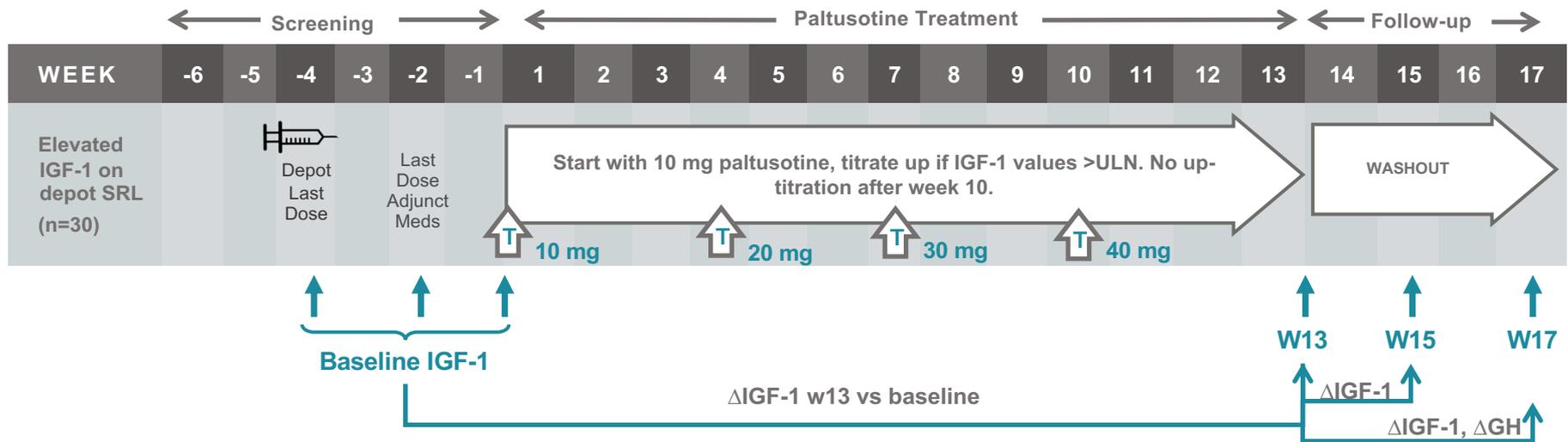
This presentation contains forward-looking statements. All statements other than statements of historical facts contained in this presentation are forward-looking statements, including statements regarding: the potential for interim data results to be replicated or continue to show clinical efficacy as the ongoing trial continues; the potential benefits of paltusotine for acromegaly patients; the potential to initiate a pivotal Phase 3 trial of paltusotine in acromegaly based on interim results to date and the timing thereof; the planned expansion of the paltusotine development program to include the treatment of patients with NETs and the expected timing thereof; the anticipated timing of topline data for Edge and PK/PD data for its other development programs and initiation of trials thereafter; and the company's anticipated cash runway. In some cases, you can identify forward-looking statements by terms such as "may," "believe," "anticipate," "could," "should," "estimate," "expect," "intend," "plan," "project," "will," "forecast" and similar terms. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, without limitation: the risk that interim results of a clinical trial do not necessarily predict final results and that one or more of the clinical outcomes may materially change as patient enrollment continues, following more comprehensive reviews of the data, and as more patient data become available; potential delays in the commencement, enrollment and completion of clinical trials and the reporting of data therefrom; the COVID-19 pandemic may disrupt our business and that of the third parties on which we depend, including delaying or otherwise disrupting our clinical trials and preclinical studies, manufacturing and supply chain, or impairing employee productivity; our dependence on third parties in connection with product manufacturing, research and preclinical and clinical testing; the success of our clinical trials and nonclinical studies for paltusotine and our other product candidates; regulatory developments in the United States and foreign countries; unexpected adverse side effects or inadequate efficacy of our product candidates that may limit their development, regulatory approval and/or commercialization; we may use our capital resources sooner than we expect; and other risks described under the heading "Risk Factors" in documents we file from time to time with the Securities and Exchange Commission. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, you should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. All forward-looking statements are qualified in their entirety by this cautionary statement, which is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and, except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions, and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

Corporate update

- Paltusotine Phase 2 acromegaly program achieves key milestones
 - Interim results from Edge patients who completed participation by Feb 23, 2020 showed that after 13 weeks, patients who were switched to once daily oral paltusotine maintained IGF-1 levels that were achieved with prior injected peptide octreotide or lanreotide depot monotherapy
 - Edge recruitment is now complete (topline data expected in 4Q2020)
 - New enrollment in the Evolve study has been discontinued. Patients already enrolled will continue in the study. Data from these patients are expected to be available for end of Phase 2 regulatory interactions at the same time as data from Edge.
- NETs development will proceed with paltusotine to accelerate the program and conserve resources. CRN01941 development has been discontinued.
- Cash runway guidance extended into 2022
- Acromegaly Phase 3 expected to start 1H2021 with to-be-marketed formulation of paltusotine
- ACTH antagonist and sst5 agonist programs continue with IND enabling activities with a goal of reporting of Phase 1 PK/PD proof-of-concept data in the first half of 2021

ACROBAT Edge design



Group	Patient Groups	IGF-1 range	# of Patients
1	Octreotide LAR or lanreotide depot	> 1.0x ULN to ≤ 2.5x ULN	At least 30
2	Dopamine agonist + octreotide LAR or lanreotide depot	> 1.0x ULN to ≤ 2.5x ULN	
3	Dopamine agonist + octreotide LAR or lanreotide depot	≤ 1.0x ULN	Max 15
4	Pasireotide LAR	≤ 1.0x ULN	
5	Pegvisomant + octreotide LAR or lanreotide depot	≤ 1.0x ULN	

Key inclusion/rescue criteria

- Patients on stable approved monthly dose of SRL for at least 3 mo.
- 18 to 75 years of age
- Criteria for rescue with standard acromegaly medication: significant worsening of acromegaly symptoms

EDGE interim data cut as of February 23, 2020

Data available for interim analysis

- As of February 23, 2020, 32 patients have enrolled in the study and either completed participation, or continue to receive paltusotine
- 17 patients have completed participation in the study.
 - 13 of these initial patients were treated with lanreotide depot (n=8) or octreotide LAR (n=5) monotherapy and entered the trial with IGF-1 above the upper limit of normal (Group 1)
- Efficacy Evaluation Set (n=13)
 - All available data from Group 1 patients who completed participation in the trial as of February 23, 2020
- Safety Evaluation Set (n=32)
 - All patients dosed in all subgroups as of February 23, 2020 (n=32) including those that had not yet completed the study

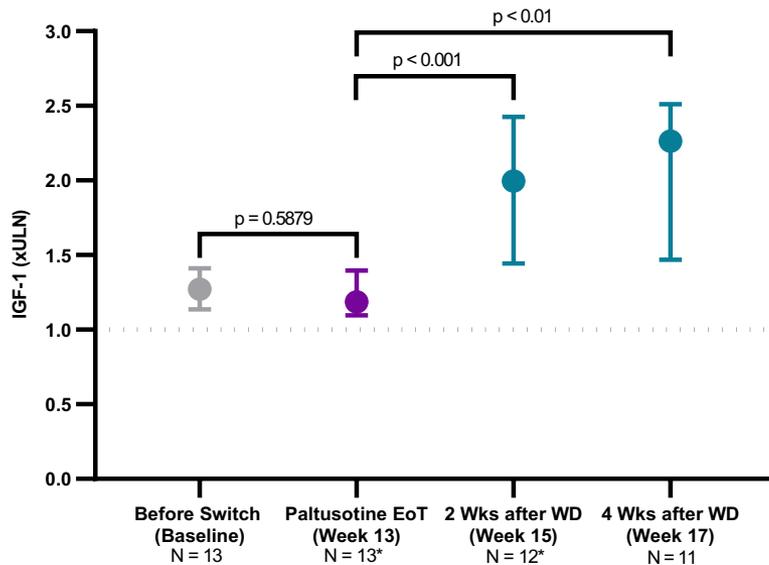
Patient baseline characteristics

	Group 1 (N=13)	Total (N=32)
Median Age, years (Min, Max)	53.0 (34, 68)	51.5 (34, 70)
Sex		
Female	7 (53.8%)	19 (59.4%)
Male	6 (46.2%)	13 (40.6%)
Ethnicity		
Hispanic or Latino	0	6 (18.8%)
Not Hispanic or Latino	13 (100%)	26 (81.3%)
Race		
White	13 (100%)	29 (90.6%)
Black or African American	0	1 (3.1%)
Other	0	2 (6.3%)
Median Weight, kg (Min, Max)	97.9 (63, 155)	89.3 (57.3, 155)

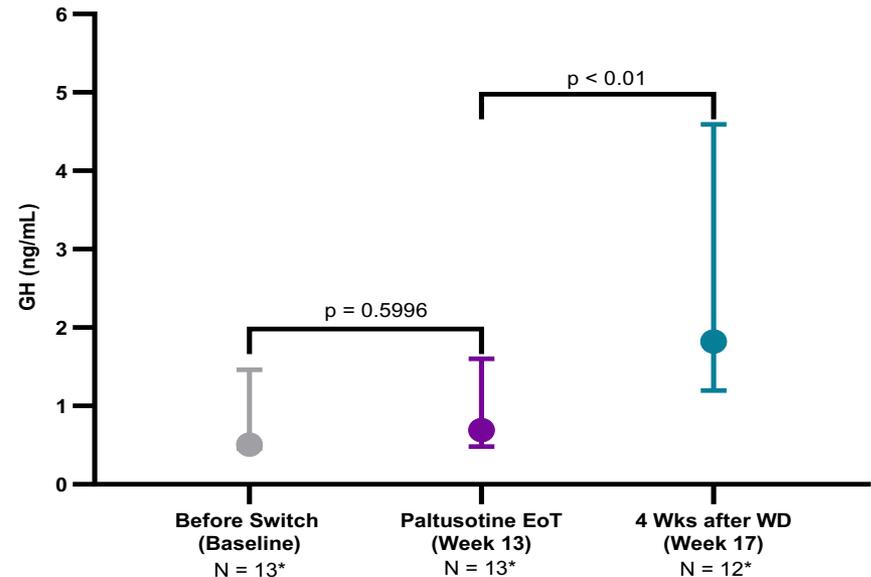
Paltusotine maintained baseline IGF-1 and integrated GH levels 13 weeks after switching from injected depot therapy

Both IGF-1 and GH levels promptly rose after paltusotine withdrawal

Serum IGF-1 changes at end of treatment and after withdrawal of paltusotine



Serum GH changes at end of treatment and after withdrawal of paltusotine



Average of 3 GH values drawn within 2 hours

Data shown are median (25th percentile, 75th percentile) for Group 1 patients

- *Data includes two early termination (ET) patients who discontinued for non-study drug related reasons: (1) use of prohibited concomitant medication and (2) inability to complete study visits. Their final treatment values were used for EoT. The 2 ET patients had 16 days (~2 weeks) and 23 days (~3 weeks) of paltusotine exposure. One ET patient had final assessment 2 weeks after EoT (GH data from this subject was included in the 4 wk GH WD data), the washout data for the other was not available
- p-values are based on non-parametric Wilcoxon Sign Rank test of whether the median change is different from zero.
- EoT=End of Treatment; WD=Withdrawal

Exploratory statistical analysis of Group 1 patients



Efficacy Conclusions

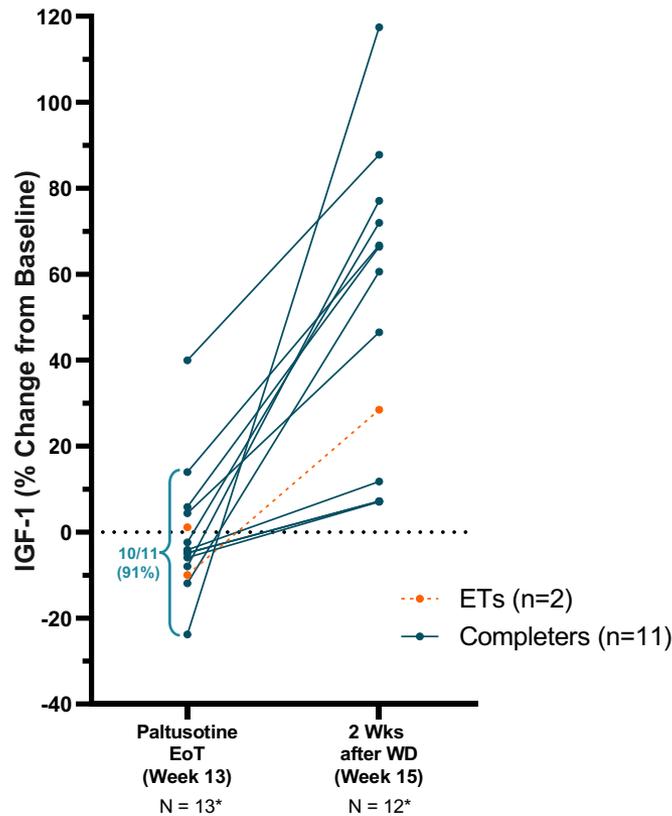
1. IGF-1 and GH levels after 13 weeks of paltusotine treatment were not different than baseline (while treated with SRL depot)
2. IGF-1 rose significantly within 2 weeks of paltusotine withdrawal. GH hormone also rose significantly (GH measured only 4 weeks after withdrawal)

Parameter (units)	Change in Serum Hormone Levels		
	Change from Baseline to EoT (Week 13)	Change from EoT to Post Withdrawal Visit	
		2 Weeks	4 Weeks
IGF-1 (×ULN)	N=13	N=12	N=11
Mean (95% CI)	-0.015 (-0.123, 0.092)	0.739 (0.394, 1.083)	0.767 (0.379, 1.155)
Median (25 th , 75 th percentiles)	-0.047 (-0.102, 0.050)	0.574 (0.297, 1.083)	0.782 (0.371, 1.250)
p-value [^]	0.5879	< 0.001	< 0.01
GH (ng/mL)	N=13	Not Measured	N=12
Mean (95% CI)	0.054 (-0.285, 0.394)		1.612 (0.452, 2.772)
Median (25 th , 75 th percentiles)	0.011 (-0.100, 0.240)		0.891 (0.358, 2.716)
p-value [^]	0.5996		< 0.01

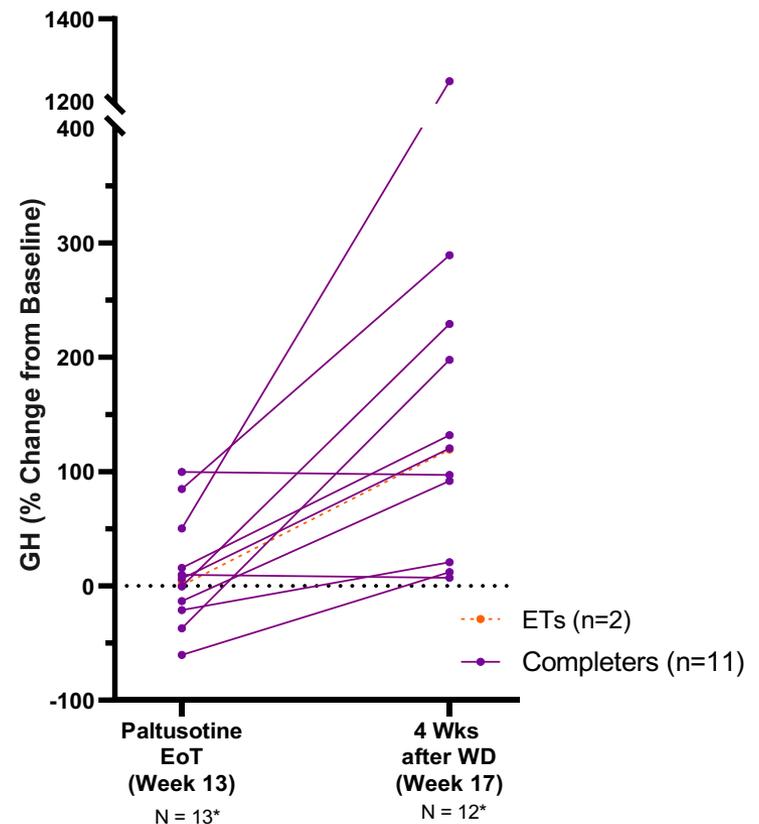
[^]p-values are based on non-parametric Wilcoxon Sign Rank test
EoT=End of Treatment;

Switching to once daily oral paltusotine from injected depot SRLs maintained IGF-1 levels in > 90% of Group 1 completers

Individual IGF-1 changes at end of treatment and 2 weeks after withdrawal of paltusotine



Individual GH changes at end of treatment and 4 weeks after withdrawal of paltusotine



- EoT=End of Treatment; WD=Withdrawal
- *Data includes two early termination (ET) patients who discontinued for non-study drug related reasons and had 16 days (~2 weeks) and 23 days (~3 weeks) of paltusotine exposure. 1 ET patient had final assessment 2 weeks after EoT (GH data from this subject was included in the 4 wk GH WD data), the washout data for the other was not available. These were not included in calculation of 91% who maintained IGF-1 levels within 15% of baseline values.
- Pre-trial therapy SRL median concentrations at W13 compared to baseline: [Lanreotide] ↓ by 70%; [Octreotide] ↓ by 100%; paltusotine median concentrations at W15 compared to W13 (last dose) were decreased by >99.9%

Paltusotine was safe and well tolerated



Safety data in all patients dosed with paltusotine as of February 23, 2020 (n=32) in Edge

Adverse events on treatment regardless of causality in 2 or more patients

Preferred Term	Group 1 (N=13) n (%)	Total (N=32) n (%)
Number (%) of Patients with any TEAEs	9 (69%)	14 (44%)
Arthralgia	3 (23%)	6 (19%)
Headache	4 (31%)	5 (16%)
Abdominal discomfort	1 (8%)	3 (9%)
Peripheral swelling	2 (15%)	3 (9%)
Back pain	2 (15%)	2 (6%)
Diarrhoea	0	2 (6%)
Flatulence	0	2 (6%)
Hyperhidrosis	2 (15%)	2 (6%)
Palpitations	1 (8%)	2 (6%)

- No discontinuations due to adverse events
- No patients have required “rescue treatments” with standard acromegaly medications
- 1 SAE--Headache--non-treatment related (admission for diagnostic evaluation)
- No safety signals to date with vital signs, clinical safety laboratories (including amylase/lipase, fasting glucose, liver function tests), HbA1c, ECGs
- Acceptable safety and tolerability profile has also been shown in >100 healthy volunteers dosed with paltusotine to date