calliditas
THERAPEUTICS

Phase 3 Part A Topline Data

November 2020

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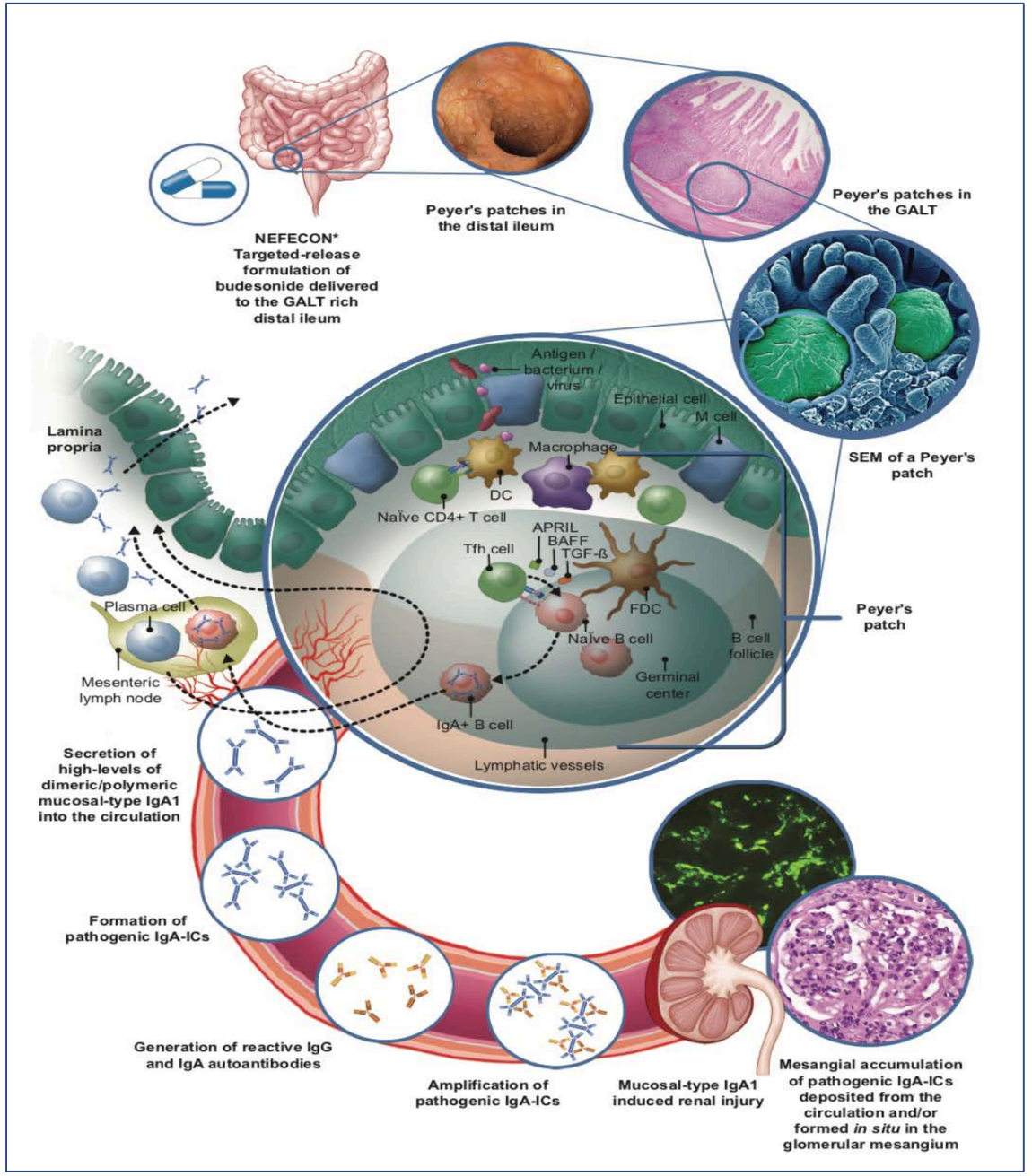
Company Overview

1. Calliditas is a clinical-stage biopharma company focused on novel treatments in orphan indications. **Cash balance** as of June 30, 2020 of US$169M, provides financing through **Q3 2022**
2. Lead candidate Nefecon is a proprietary, novel investigational treatment for IgAN intended to be **disease modifying**
3. Nefecon targets the presumed **origin** of the disease – the area of the ileum where the highest concentration of Peyer's patches is located
4. Nefecon is the **most advanced** product candidate for IgAN, and is positioned to be the **first approved drug** specifically designed for IgA Nephropathy
5. Calliditas has carried out the **only successful** randomized, double-blind, placebo-controlled Phase 3 clinical trial in IgAN
6. Regulatory pathway based on FDA and EMA acceptance of accelerated / conditional approval based on proteinuria as **surrogate marker** for IgAN
7. **Significant unmet medical need** in IgAN with no currently approved treatments; total market opportunity of US$9-10bn in the U.S alone

IgA Nephropathy

Mechanism of Action

- Follicles of lymphatic tissue, known as Peyer's patches, in the distal part of the small intestine (ileum) produce secretory IgA antibodies
- Patients with IgAN have an increased appearance in the blood of secretory IgA antibodies that lack galactose units (galactose-deficient IgA) in the hinge region, which makes the antibody immunogenic
- These IgA antibodies trigger autoantigen production and form autoantibody complexes with autoantibodies directed against the IgA hinge region
- These aggregates form pathogenic immune complexes that deposit in the glomeruli, which are the filtration apparatus of the kidney
- The deposits of immune complexes in the glomeruli cause an inflammatory cascade that destroys the glomeruli, reducing the kidney's ability to remove waste products from the blood and eventually may result in ESRD



Nefecon: Disease-Modifying Investigational Drug for IgAN

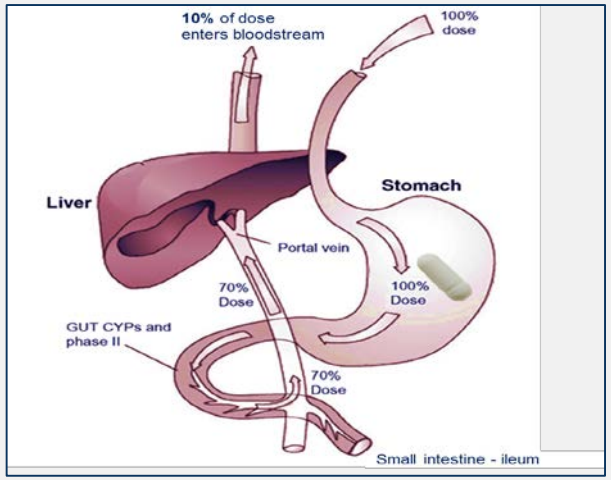
Nefecon

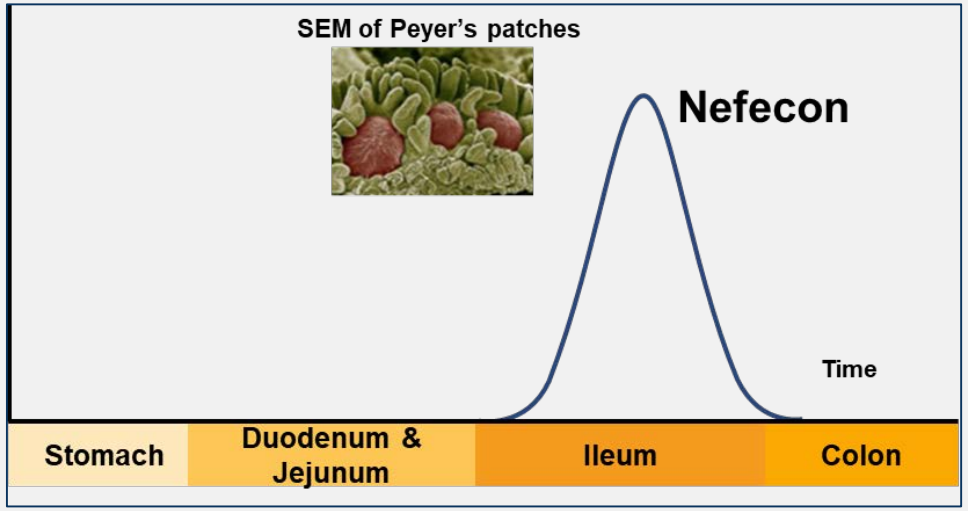
Drug product based on known active ingredient

- Active ingredient is budesonide – an established, highly potent, locally acting corticosteroid
- 90% cleared in first pass metabolism by liver, which minimizes systemic side effects

Novel targeted release profile

- Designed to deliver a targeted and highly concentrated dose of budesonide directly to the Peyer's patches in the ileum
- Differentiated release profile
  - pH-governed delayed disintegration of the capsule until it reaches the ileum
  - Potent, sustained exposure throughout the ileum



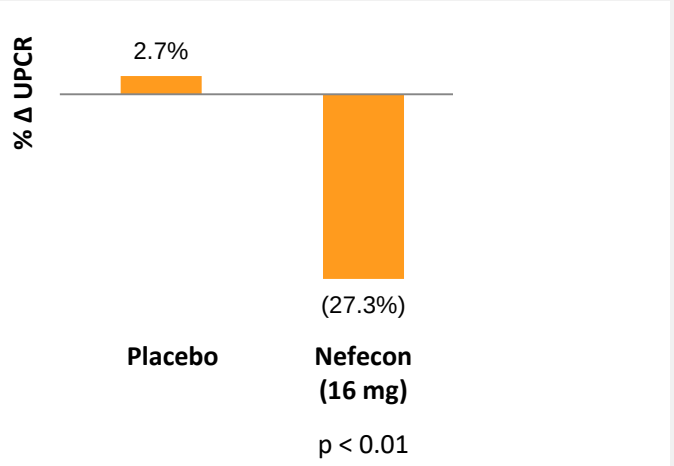


Successful Phase 2b trial

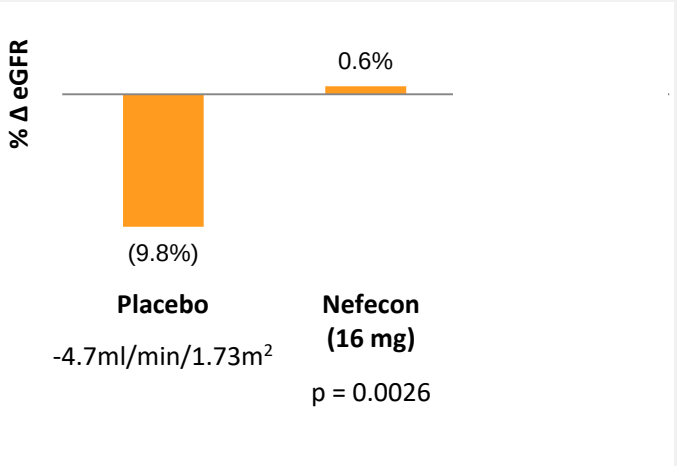
Only Phase 2b Study in IgAN to reach Primary Endpoint

- Large study population – 150 patients
- Oral dose taken daily over a nine-month period
- Randomized, double-blinded, placebo controlled
- European study in 62 sites in 10 countries

Primary endpoint: Reduction in proteinuria



Secondary endpoint: Stabilization of eGFR



Study results published in The Lancet, 2017

Efficacy Findings

- ✓ Phase 2b trial of 150 patients demonstrated statistically significant and clinically meaningful reduction in proteinuria and eGFR stabilization in the 16 mg dose cohort
- ✓ Statistically significant UPCR reduction with Nefecon (16 mg) compared to placebo – 9 months treatment ($p<0.01$)
- ✓ Statistically significant eGFR stabilization with Nefecon (16 mg) compared to placebo – 9 months treatment ($p=0.0026$)

Tolerability Findings

- ✓ Generally well-tolerated, with two possibly treatment-related serious adverse events
- ✓ Treatment-related adverse effects were transient and mainly mild (75.8%) to moderate (22.6%); consistent with those known to be associated with non-systemic corticosteroids
- ✓ No material metabolic adverse events (hypertension, diabetes, weight gain)
- ✓ No severe infections

Trial Design: Phase 2b -> Phase 3

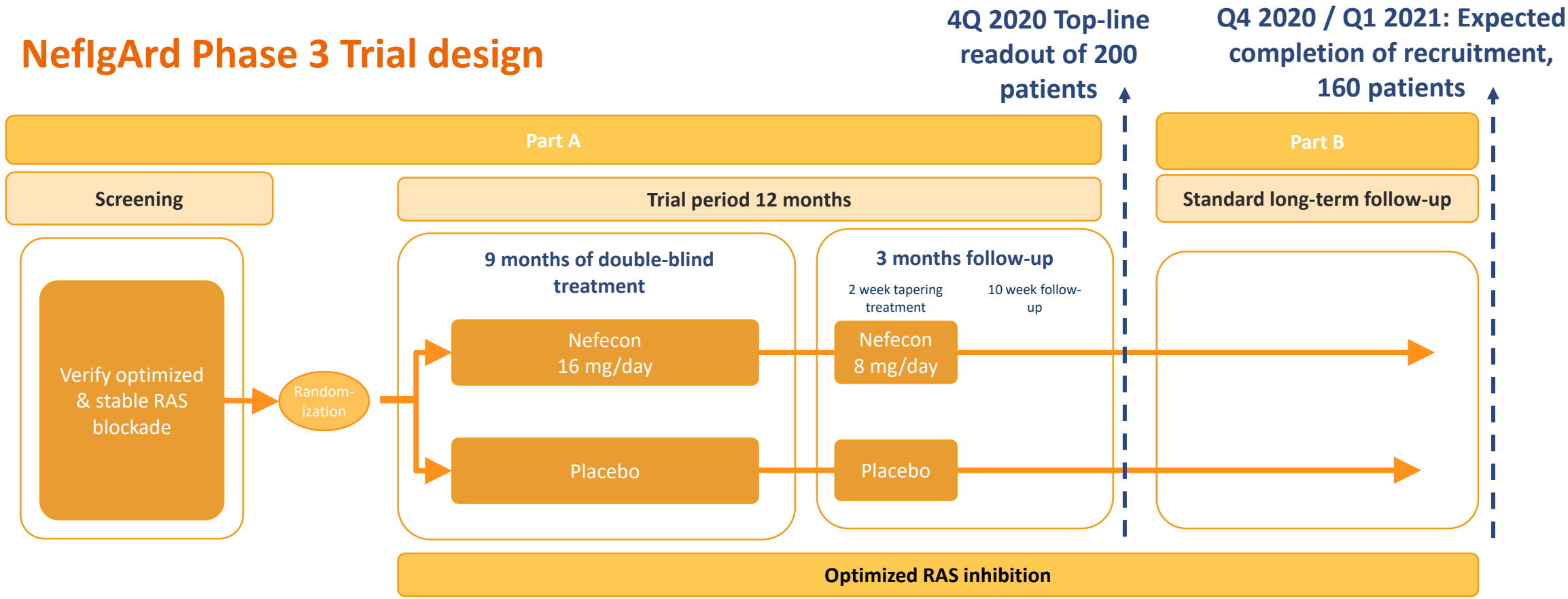
Phase 2b Trial Design	Phase 3 Trial Design
Large trial population –150 patients	Large trial population – 200 patients in Part A
Oral dose taken daily over a nine-month period	Oral dose taken daily over a nine-month period
Randomized, double-blinded, placebo-controlled	Randomized, double-blinded, placebo-controlled
European trial in 62 sites in 10 countries	Global trial in 146 sites in 19 countries
Primary endpoint: Reduction in proteinuria	Primary endpoint: Reduction in proteinuria
Key secondary endpoint: Stabilization of eGFR	Key secondary endpoint: Stabilization of eGFR

Pivotal Phase 3 clinical trial (NefIgArd) designed to confirm Phase 2b results

Key highlights

- → Reduction of proteinuria: Accelerated approval in U.S. and conditional approval in E.U. based on Part A data
- → Full approval; 2-year eGFR based endpoint – 360 patients

NefIgArd Phase 3 Trial design



Phase 3 trial design & criteria

- Two-part trial design based on feedback from FDA and EMA:
  - **Part A**
    - N=200, two arms;
      - 16mg Nefecon and placebo
      - global trial in 19 countries and approximately 146 sites
    - Primary endpoint: decrease in UPCR at 9 months (same as Phase 2b trial)
    - Fully recruited as of Dec. 2019
  - **Part B**
    - N=360 (including Part A patients)
    - Primary endpoint: difference in kidney function between treated and placebo patients as measured by eGFR over a 2-year period from dosing (same metric as Part A's key secondary endpoint over a 1-yr period)
    - To confirm the long-term clinical benefit of observed proteinuria reduction
    - Completion of recruitment of 360 patients is expected in Q4 2020 or Q1 2021, depending on the impact of COVID-19
    - Readout 2 years after last patient is randomized

Inclusion criteria

- Biopsy-confirmed primary IgA Nephropathy (IgAN)
- ≥18 years
- Total urine protein ≥ 1g / day (KDIGO guidelines)
- eGFR ≥ 35 mL/min * 1.73m^2 and ≤ 90 ml/min * 1.73m^2
- Stable RAS treatment for 3 months

Exclusion criteria

- Secondary forms of IgAN
- TB
- Kidney transplanted patients
- Treatment with high dose corticosteroids or immunosuppressants in the past 12 months for the treatment of IgAN

Main differences from Phase 2b

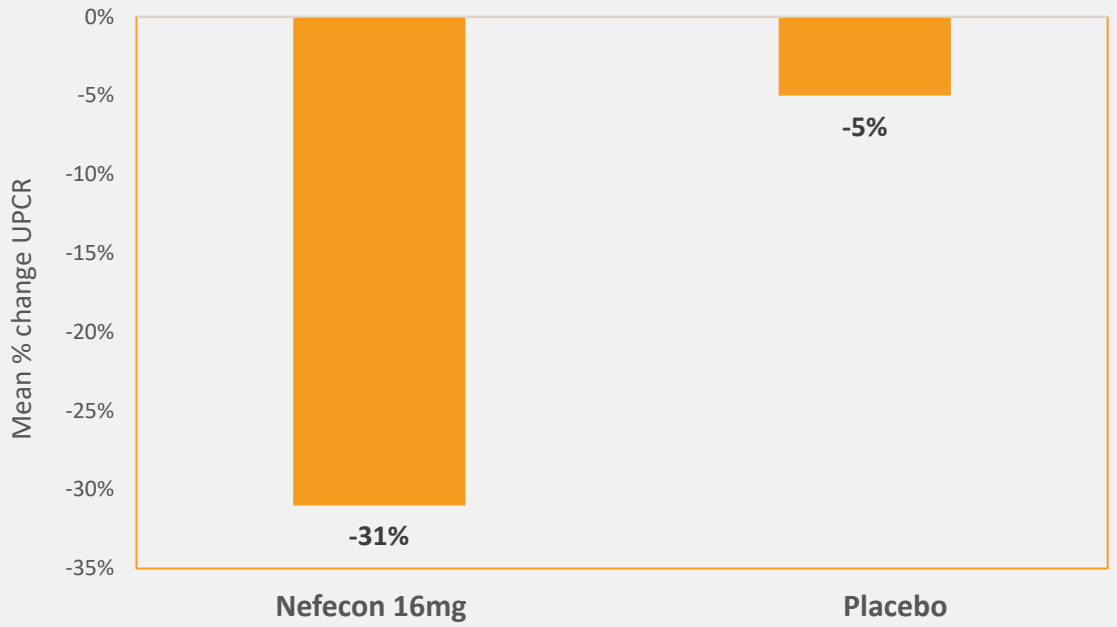
- Aligning to KDIGO guidelines - slightly more severe patients in phase 3
  - *Total urine protein from ≥0.75 g/day to 1g/day*
  - *eGFR from > 45 to ≥35 and ≤90 mL/min/1.73m^2*

NefIgArd Phase 3 Part A: Topline data - Primary Endpoint

Efficacy findings: UPCR at 9 months

- ✓ Phase 3 study demonstrated a reduction in mean UPCR from baseline:
  - 31% reduction in the 16mg Nefecon arm
  - 5% reduction in the placebo arm
- ✓ **Statistically significant treatment effect of 27%** UPCR reduction of Nefecon (16 mg) compared to placebo at 9 months (p=0.0005)

Primary endpoint: Reduction in proteinuria

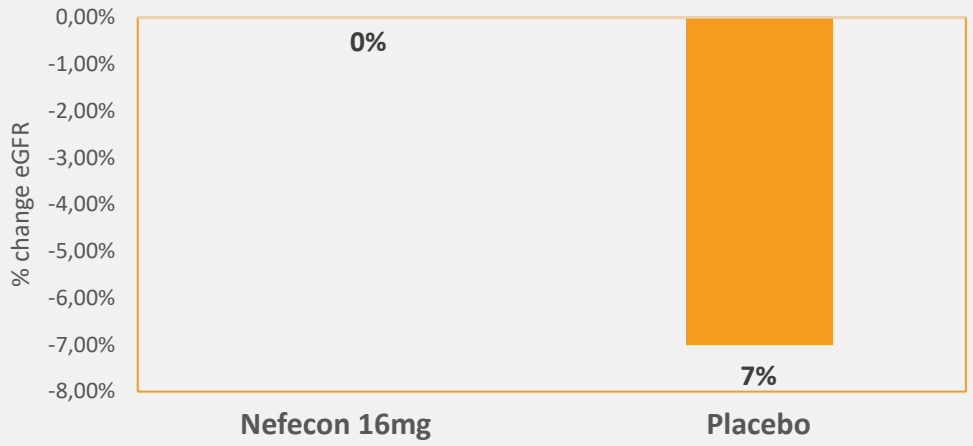


NefIgArd Phase 3 Part A: Key Secondary Endpoint and Safety Profile

Efficacy findings: eGFR at 9 months

- ✓ Phase 3 study demonstrated significant treatment effect on eGFR
  - ✓ Mean reduction in eGFR of 7% in the placebo arm versus baseline
  - ✓ Treatment arm stable
- ✓ Statistically significant eGFR stabilization with Nefecon (16 mg) compared to placebo – 9 months treatment (p=0.0029)

Key Secondary endpoint: eGFR



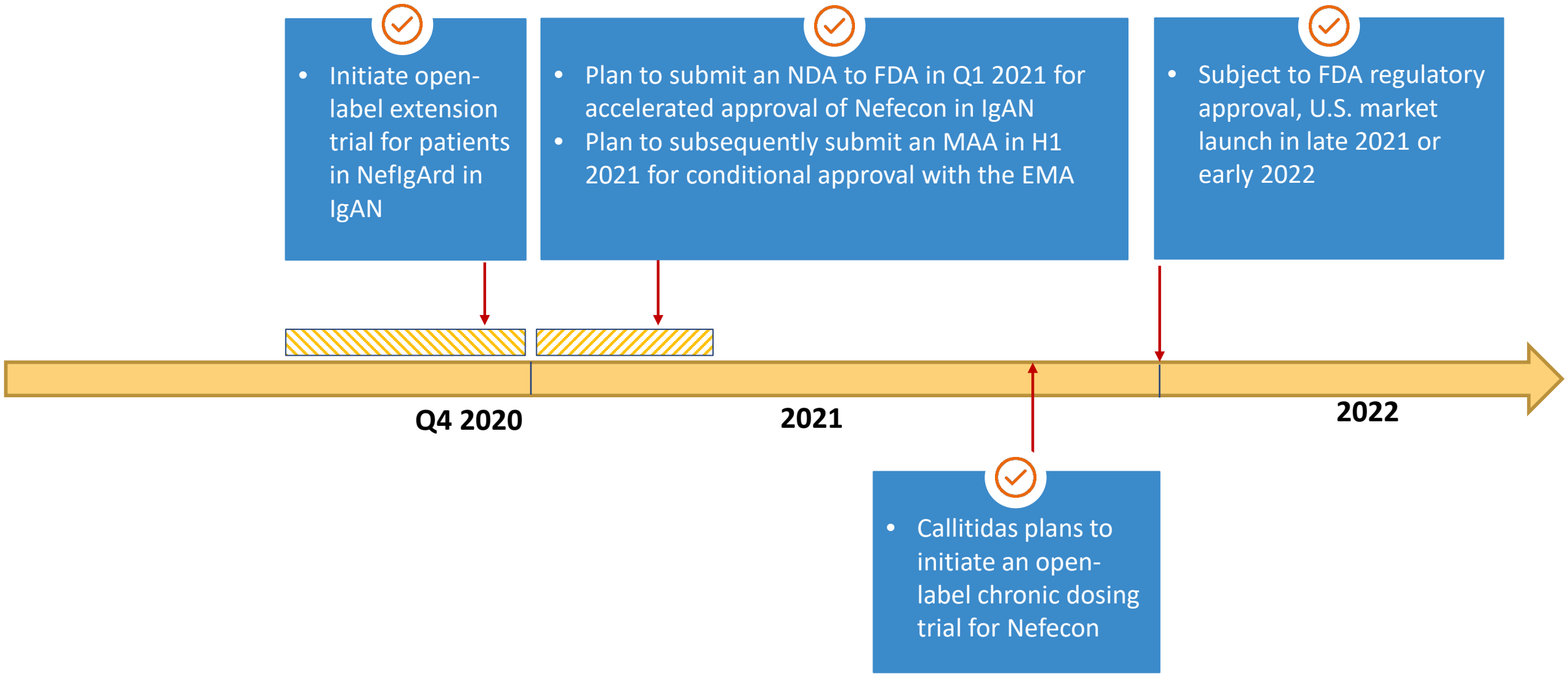
Absolute Difference: **-0.17** ml/min/1.73m^2 **-4.04** ml/min/1.73m^2

Tolerability findings

- ✓ Generally well-tolerated, with a safety profile that is consistent with known active ingredient
- ✓ No severe infections
- ✓ Significantly less withdrawals compared to Phase 2b

Next Steps

- Callidtas' Nefecon development and regulatory program overview



Part B

- Post-approval trial to confirm long term renal protection
- 2 year eGFR based endpoint

Primary outcomes

- eGFR over 2 years endpoint[1,2]
- Based on the 2018 NKF/FDA/EMA workshop[3]

Secondary outcomes[2]

- Time to 30% reduction from baseline in eGFR (CKD-EPI)
- Time to rescue medication
- Ratio of UPCR, UACR, and eGFR (CKD-EPI) compared with baseline*
- Proportion of patients without microhematuria
- Descriptive safety outcomes
- SF-36 quality of life assessment

- Recruitment is on-going, with completion of recruitment expected in Q4 2020 or Q1 2021
  - Timelines potentially affected due to recent deterioration of COVID-19 pandemic
- Everest Medicines, Calliditas' partner in China, is contributing patients to Part B of the trial
  - First patient was randomized in China on 8 September 2020

*Averaged over time points between 12 and 24 months, inclusive, following the first dose

CKD-EPI, Chronic Kidney Disease-Epidemiology Collaboration; NKF, National Kidney Foundation; SF-36, Short Form 36; UACR, urine albumin-creatinine ratio

1. Barratt J *et al. Kidney Int Rep* 2020;5:1620; 2. Calliditas Therapeutics. Data on File. 2019; 3. Levey AS *et al. Am J Kidney Dis* 2020;75:84