# Calliditas Therapeutics Q2 Report 2018

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Presenters:

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# Calliditas Therapeutics in brief

Company overview

- Calliditas is a specialty pharmaceutical company focused on developing high value pharmaceutical products for orphan diseases with a lead program for patients with IgA nephropathy – an orphan chronic autoimmune disease
- Lead product candidate Nefecon is an optimized oral formulation of budesonide with a unique and disease specific dosing and release profile
- Clinical Phase 3 study NEFIGARD ready to start recruiting in H2 2018
- Only successful placebo controlled Phase 2b study to date (150 patients). Approval phase of NEFIGARD financed after successful IPO in June 2018
- Headquartered in Stockholm, 19 co-workers (10 employees and 9 consultants)

# Summary of key events Q2 2018

- Successful roadshow and completion of IPO
  - SEK 650m raised on main market of Nasdaq Stockholm, midcap list
  - Market cap approx. SEK 1.8bn, August 13, 2018
- Substantially oversubscribed with interest mainly from the Nordics, the UK and the US
- Continued work with preparations for the Phase 3 study NEFIGARD
  - Over 90% of study sites identified
  - Study substance completed and available
  - The Phase 3 protocol has now been approved in 11/19 countries.

# Post period highlights

- Over-allotment option exercised providing the company with an additional SEK 89m of proceeds before issue expenses
- Selected for presenting at *NewsMaker in the Biotech Industry* conference sponsored by BioCentury in NY on September 7
  - Important conference for US life sciences investors
- Five abstracts accepted by the IIgANN conference in Buenos Aires, Argentina, on September 27-29
  - Conference marks the 50 year anniversary of the initial description of the disease by Dr. Berger and Dr. Hinglais
- Ann-Kristin Myde has taken on the role of VP of Development, retaining her overall Project management role

# Our main indication: IgA nephropathy – large unmet medical needs

## PROFILE

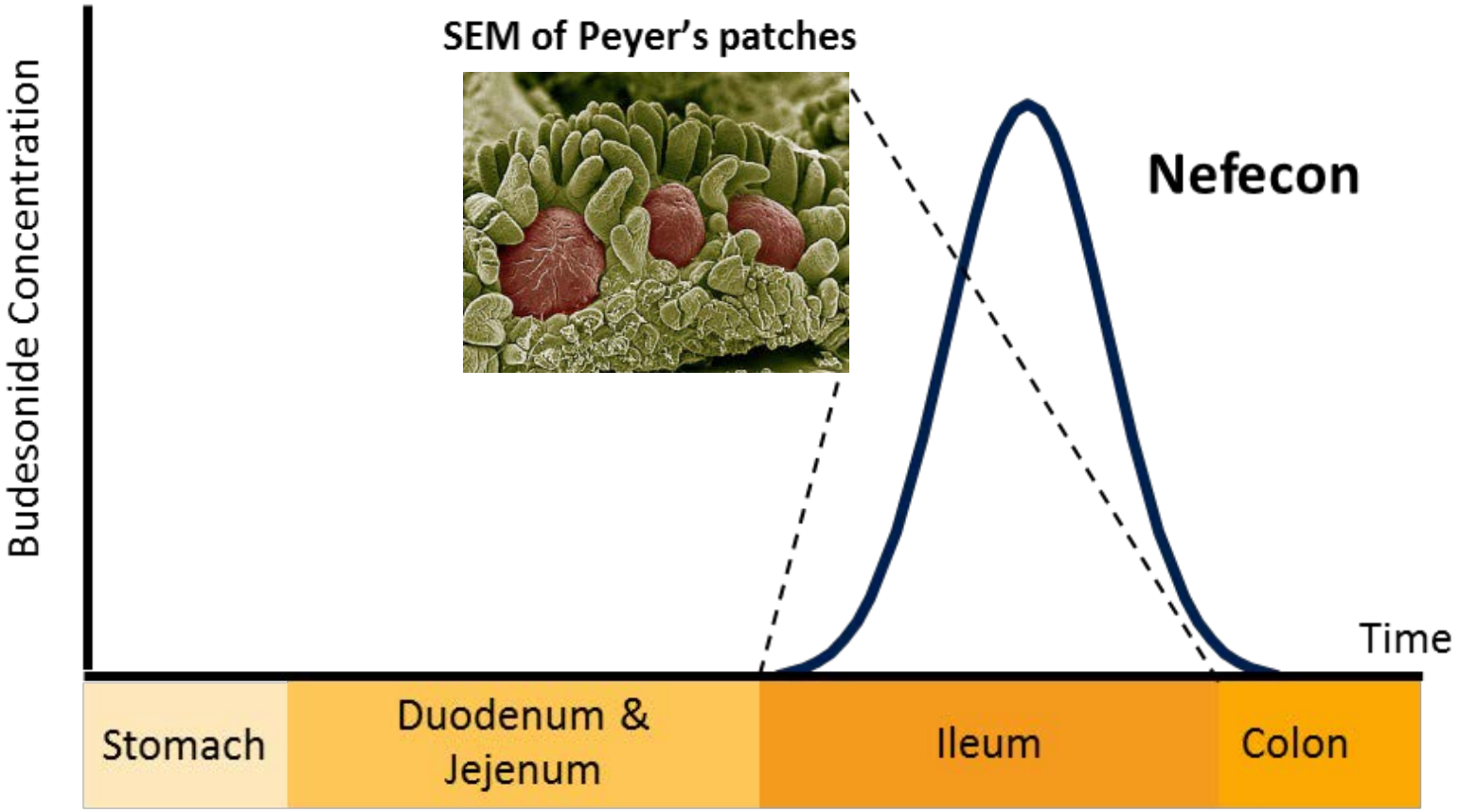
- Genetic predisposition – not sufficient but necessary. Environmental, bacterial, dietary triggers.
- Incidence estimated at 2.5 per 100,000 - For the US market corresponding to approximately 6,000-7,000 new cases each year
- Normally presents in the 20-30s – more prevalent in men than in women

## ESTIMATED PREVALENCE

| | | |
|---|---|---|
| MAIN MARKET |  | **130,000-150,000** |
| |  | **200,000** |
| POTENTIAL MARKET OPPORTUNITIES |  | **~2,100,000** |
| |  | **~190,000** |

# Key properties of our lead candidate Nefecon

Release profile of Nefecon




Comments

- Targeted local delivery of potent immunosuppressive agent to Peyer's patches in the ileum
- 90% first pass liver metabolism → minimize systemic side effects
- Substantially similar design to successful large Phase 2b study → significantly reduced development risk
- Unique two-step release profile
  - PH-governed delayed disintegration of the capsule
  - Sustained but fast uptake throughout the Ileum

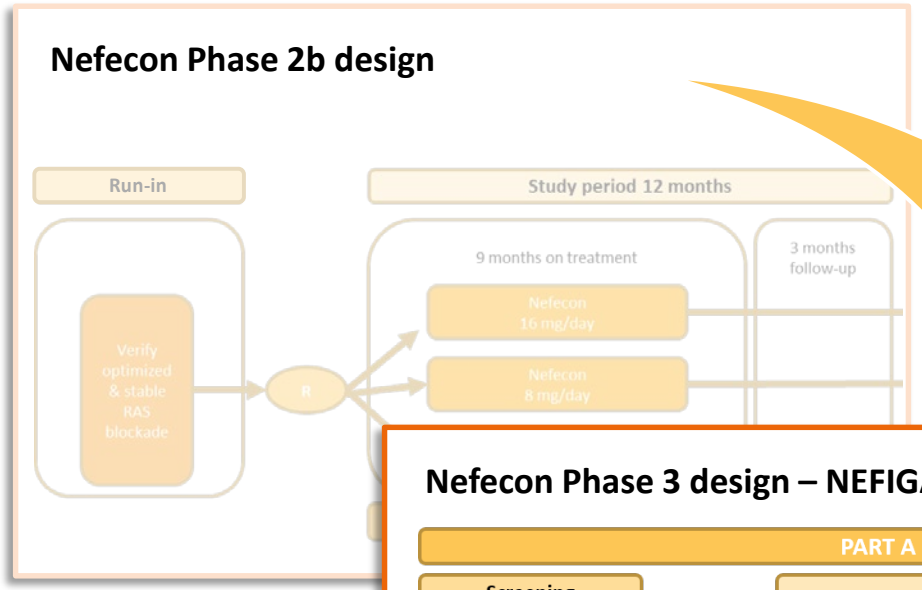
# Development program is regulatory agreed and de-risked

Proteinuria – Accepted by FDA as surrogate marker for Phase 3 and accelerated approval…

…supported with post-approval outcome data based on eGFR endpoint

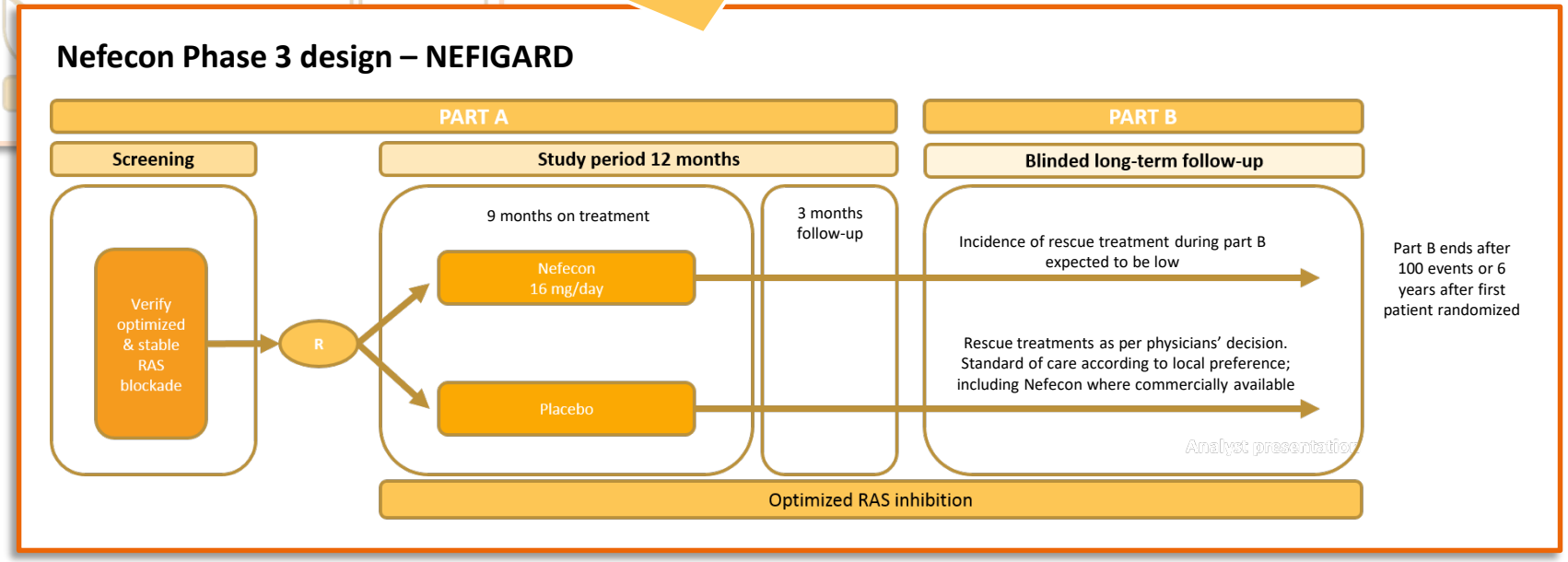
- → Clear strategy for the further development and approval of Nefecon from end of Phase 2b meetings
- → The first company to receive acceptance by the FDA to use proteinuria as Phase 3 endpoint for approval
- → The opportunity to become the first drug to be approved for broad use in this indication – safe, efficient and convenient
- → FDA and all major European countries have accepted Phase 3 design and protocol

# Clinical Phase 3 study NEFIGARD to confirm Phase 2b results




**Key highlights**

- → Phase 3 study design replicates successful Ph2b
- → Only 200 versus previous 150 patient population (Phase 2b)
- → Fixed 16mg Nefecon once daily oral dose
- → Recognized surrogate marker for approval



# Study aim: to achieve proteinuria reduction in IgAN patients

→Approximately 150 clinical sites in 19 countries
→450 patients

→Read out on first 200 patients basis for approval and US market launch

→Time to top line read-out: Part A estimated H2 2020

→Endpoint: Proteinuria reduction is the surrogate marker for approval of Phase 3 study…

→…with eGFR as a post approval endpoint

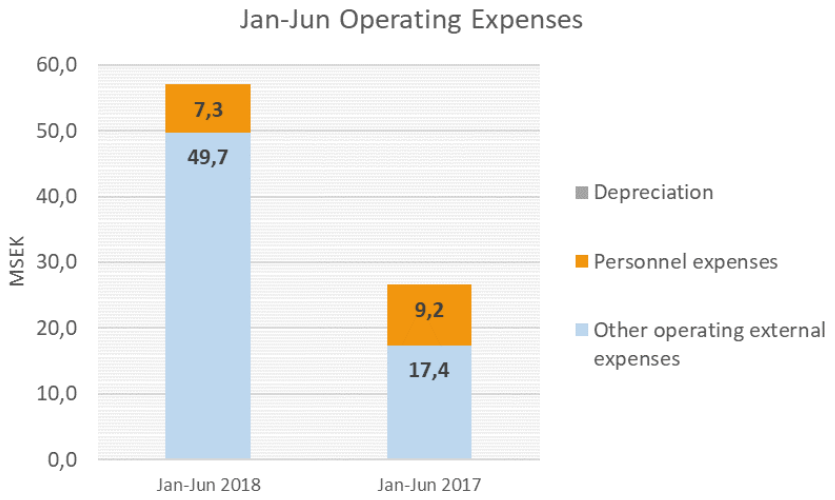
- Endpoint for Phase 3 study same as in Phase 2b - **proteinuria reduction** measured in the first **200 patients** after **nine months** of treatment – basis for the accelerated approval in the US /conditional in Europe
- Potential for full approval if proteinuria reduction is substantial, or based on results from interim analysis of eGFR from the 450 completed patients, expected around 6 months after receiving accelerated approval
- Convenient, oral medication of well tolerated substance appropriate for broad population with potential for disease modification – avoidance of dialysis / transplantation
- Results from the **full set of patients** after long-term follow-up, including an **eGFR reduction endpoint** to support post-approval filing for full approval
- First patient is expected to enroll in second half 2018

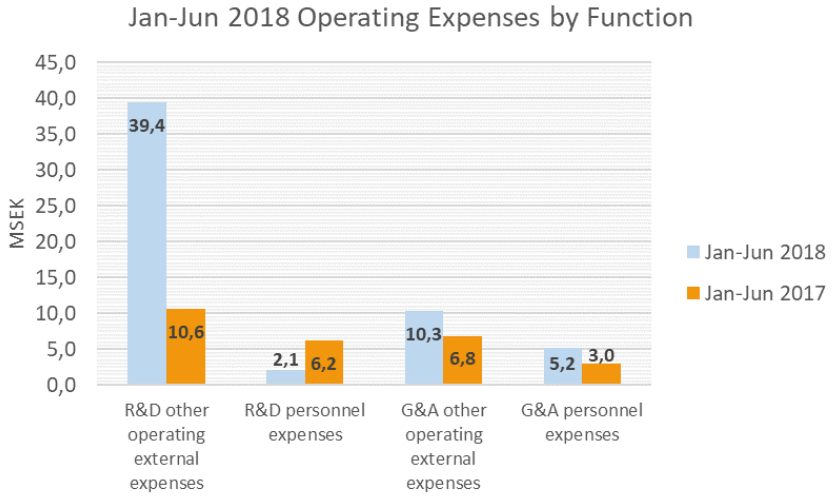
# Going forward: full focus on the Nefecon program

**Ongoing updates regarding commercial strategy and plans**

| H1 2018 | H2 2018 | H1 2019 | H2 2019 | H1 2020 | H2 2020 | H1 2021 | H2 2021 |
|---|---|---|---|---|---|---|---|
| • Filing of new patent application related to Nefecon ☑ | • NEFIGARD first patient<br>• Pediatric investigational plan submitted to EMA<br>• Application for ODD for second indication submitted | • FDA response regarding regulatory path proposal for pipeline asset<br>• Publication of new data from exploratory studies from Phase 2b in major scientific publication<br>• Approval of ODD designation for second indication | • EMA decision regarding pediatric pathway<br>• FDA / EMA meetings regarding regulatory pathway for second indication | • Part A fully recruited | • Top line data 200 patients<br>• Study fully recruited<br>• Filing with regulatory agencies | • Enrolment first patient in treatment modality trials / label expansion | • Interim analysis based on 450 patients |

# Financial overview of the period Jan-Jun 2018




- Operating loss increased to SEK -56.5 M (loss: -26.5)
  - Personnel expenses decreased to SEK 7.3 M (9.2). Credit received in Q1 of SEK 1.5 M on payroll tax.
  - Other operating expenses increased to SEK 49.7 M (17.4) primarily due to the preparations for the upcoming NEFIGARD study and to a minor extent of IPO preparations.
- Cash flow from operating activities decreased to SEK -56.4 M (-26.5).
- Cash from IPO received in July, and therefore the SEK 650 M new share issue will be reported in Q3.
- Net cash flow for 1H was SEK -40.3 M (11.4).
- The cash position per end of June 2018, was SEK 17.0 M (35.7).

# Calliditas Summary

1. Novel disease modifying treatment of IgA nephropathy (IgAN)
2. Clear path to market – FDA acceptance of proteinuria as surrogate marker
3. Mode of action targets the origin of the disease
4. Only successful Ph2b study in IgA nephropathy (150 patients)
5. Design of upcoming clinical Phase 3 study NEFIGARD replicates Phase 2b to maximize probability of success
6. Strong product protection and product exclusivity position
7. USD 1bn orphan market opportunity with no approved treatments today
8. Additional potential for Nefecon in liver disease

# Q&A

**Financial Calendar**

- Interim report for the period 1 January – 30 September 2018, 1 November 2018
- Year-end report for the period 1 January – 31 December 2018, 7 February 2019

**IR Contact**

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