# **EMPAVELI™** (pegcetacoplan) **FDA** Approval Conference Call

May 17, 2021







# **Apellis Participants**

### **CEDRIC FRANCOIS, M.D., Ph.D.**

Co-Founder & Chief Executive Officer

### FEDERICO GROSSI, M.D., Ph.D.

Chief Medical Officer

### **ADAM TOWNSEND**

Chief Commercial Officer

### **TIM SULLIVAN**

Chief Financial Officer

# Forward-looking Statements

Statements in this presentation about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute "forward-looking statements" within the meaning of The Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to, statements relating to the implications of preliminary clinical data. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Actual results may differ materially from those indicated by such forwardlooking statements as a result of various important factors, including: whether the company's clinical trials will be fully enrolled and completed when anticipated; whether preliminary or interim results from a clinical trial will be predictive of the final results of the trial; whether results obtained in preclinical studies and clinical

trials will be indicative of results that will be generated in future clinical trials; whether pegcetacoplan will successfully advance through the clinical trial process on a timely basis, or at all; whether the results of the company's clinical trials will warrant regulatory submissions and whether pegcetacoplan will receive approval from the FDA or equivalent foreign regulatory agencies for GA, PNH, CAD, C3G, IC-MPGN, ALS or any other indication when expected or at all; whether, if Apellis' products receive approval, they will be successfully distributed and marketed; and other factors discussed in the "Risk Factors" section of Apellis' Annual Report on Form 10-K filed with the Securities and Exchange Commission on April 28, 2021 and the risks described in other filings that Apellis may make with the Securities and Exchange Commission. Any forwardlooking statements contained in this press release speak only as of the date hereof, and Apellis specifically disclaims any obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

# EMPAVELI Is Approved in the United States!





First FDA-approved targeted C3 therapy

**NOW APPROVED** 

EMPAVELI is indicated for the treatment of adults with paroxysmal nocturnal hemoglobinuria (PNH)

### PNH Is a Rare, Chronic Blood Disorder

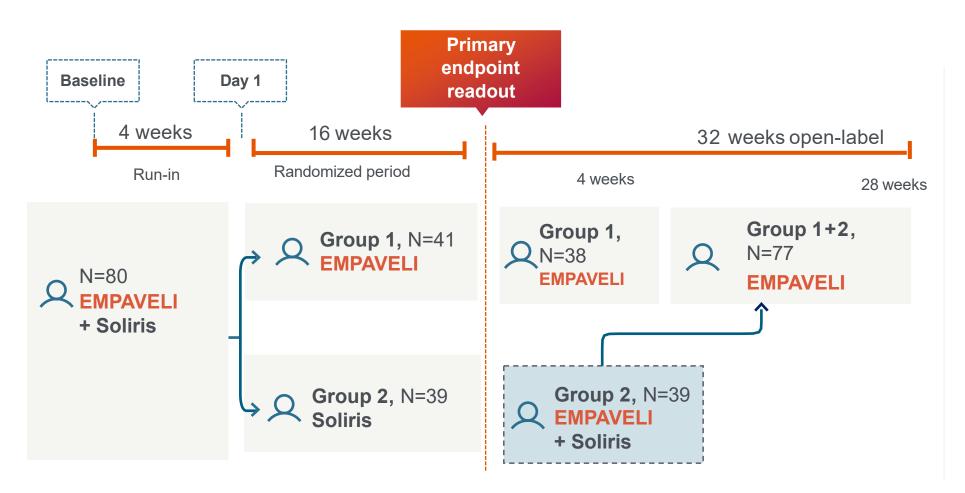
Characterized by the destruction of oxygen-carrying red blood cells through both intravascular and extravascular hemolysis<sup>1</sup>

If left untreated, PNH may lead to thrombosis and death<sup>2</sup>

Affects ~15,000 people worldwide<sup>3</sup>



# PEGASUS: Phase 3 Head-to-Head Trial of EMPAVELI vs. Soliris® (eculizumab)



Population: PNH patients who have been on Soliris (stable for at least 3 months) with a hemoglobin level of <10.5 g/dL at screening

**Primary endpoint:** Change in hemoglobin

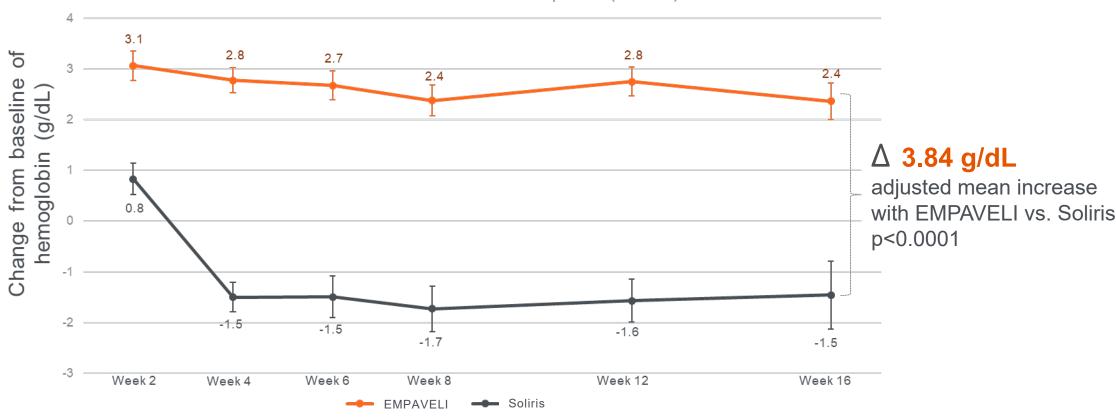
#### Secondary endpoints include:

Transfusion avoidance, change in reticulocytes, LDH, and FACIT-fatigue score

# EMPAVELI: Superior to Soliris on Improving Hemoglobin Level at Week 16 in PEGASUS

### **Met Primary Endpoint in Phase 3 PEGASUS Study**

LS Mean (± SE) plot of change from baseline in hemoglobin using MMRM model over time - randomized controlled period (ITT set)



LS, least squares; MMRM, mixed-effect model for repeated measures.

aModel (MMRM) excludes post transfusion data for patients with transfusion.

Source: Hillman B, et al. N Engl J Med, 2021 Mar 19:394(11):1039, 1037, doi: 10.1

**Apellis** 

Source: Hillmen P, et al. N Engl J Med. 2021 Mar 18;384(11):1028-1037. doi: 10.1056/NEJMoa2029073. APL2-302; NCT03500549

# PEGASUS Safety at Week 16

EMPAVELI was generally well tolerated in the PEGASUS study.

The most common serious adverse reaction in patients treated with EMPAVELI was infections. The most common adverse reactions with EMPAVELI were injection site reactions, infections, diarrhea, abdominal pain, respiratory tract infection, viral infection, and fatigue.

No cases of meningitis and no deaths were reported in patients treated with EMPAVELI.



### **EMPAVELI:** Overview of Prescribing Information

# **Indication Statement**

# EMPAVELI™ is indicated for the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH)

1,080 mg by subcutaneous infusion twice weekly using an infusion pump

# Dosing and Administration

Dosage for patients switching to EMPAVELI from C5 inhibitors

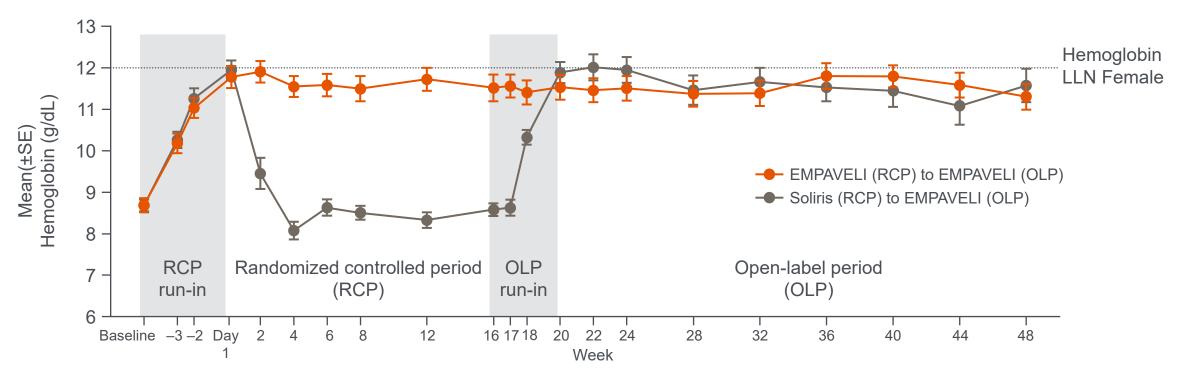
- Eculizumab: initiate EMPAVELI while continuing eculizumab at its current dose. After 4 weeks, discontinue eculizumab before continuing on monotherapy with EMPAVELI.
- Ravulizumab: initiate EMPAVELI no more than 4 weeks after the last dose of ravulizumab.

### Safety

- Boxed warning for meningococcal infections and for risk of serious infections caused by encapsulated bacteria
- REMS program requires prescriber enrollment; prescribers must educate patients and ensure vaccination
- Contraindications for hypersensitivity, lack of vaccination against encapsulated bacteria, and unresolved serious infection caused by encapsulated bacteria
- Warnings about infusion-related reactions and risk of hemolysis with discontinuation of EMPAVELI
- Potential interference with silica reagents in coagulation panels
- Most common serious adverse reaction: infections (5%)
- Most common adverse reactions (>10%): injection site reactions, infections, diarrhea, abdominal pain, respiratory tract infection, viral infection, fatigue

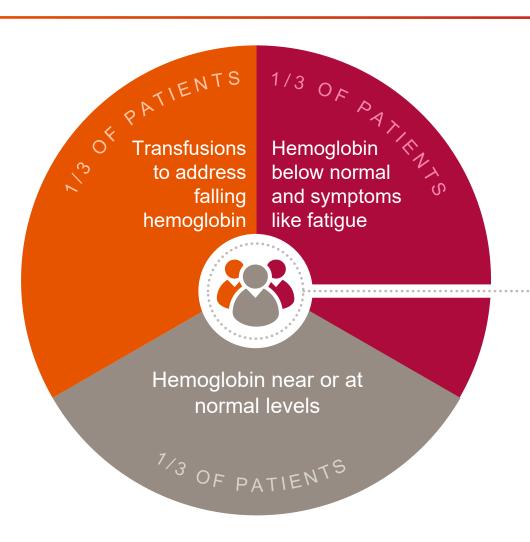
# EMPAVELI Demonstrated Sustained Improvements in Hemoglobin and Clinical Measures at Week 48

HEMOGLOBIN INCREASE FROM BASELINE AT WEEK 48 EQUAL TO INCREASE AT WEEK 16



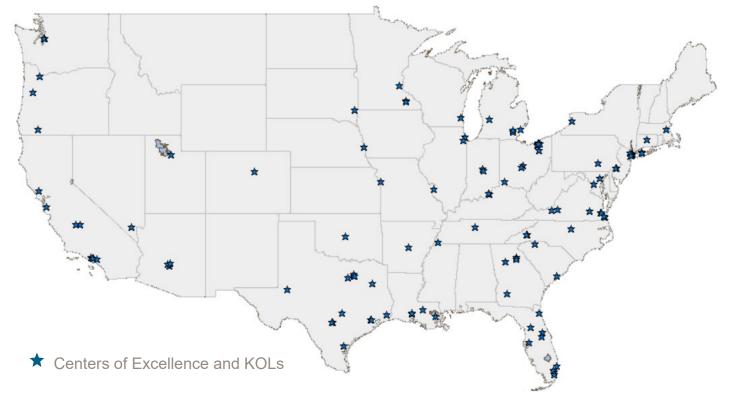
- · Sustained improvements in transfusion avoidance, reticulocyte count, LDH level, and FACIT-fatigue score
- No cases of meningitis. Safety profile consistent throughout 48-week study
- 24 of 80 EMPAVELI monotherapy-treated patients (30%) experienced a serious adverse event (SAE); 5 SAEs (6%) assessed to be possibly related to study treatment. One death reported due to COVID-19 and unrelated to study treatment

# PNH Patients on C5 Inhibitors Continue to Have High Unmet Need



1,500 U.S. PNH patients on C5 inhibitors

# Targeting the Top PNH HCPs and Treatment Centers



FOCUSED SALES TEAM DEPLOYED



1,000 – 2,000 health care professionals



More than 90 key treatment centers

### Pricing & Considerations

### **OUR PRIORITIES**



EMPAVELI demonstrated superior clinical efficacy in improving hemoglobin levels compared to Soliris in a head-to-head trial



#### **ENSURING ACCESS**

Apellis is committed to ensuring that every eligible patient who wants EMPAVELI will have access regardless of ability to pay



### **REMOVING COSTS**

We expect costs will be reduced to the healthcare system by switching patients to EMPAVELI (e.g., avoidance of transfusions)<sup>1</sup>

EMPAVELI demonstrated a superior effect on hemoglobin and reduced the transfusion burden for patients. EMPAVELI will be set at parity to Ultomiris® (ravulizumab) and at a discount to Soliris. The average annual wholesale acquisition cost, or WAC, for EMPAVELI is \$458,000.



# ApellisAssist Will Provide Support for Patients on EMPAVELI



- · Coordinate logistics for insurance approval
- Schedule monthly shipments in coordination with pharmacist
- Educate on applicable financial assistance programs
- Track insurance coverage changes and assist with coordination between HCP and insurance provider on an ongoing basis

- Live or telehealth infusion training
- Customized ongoing education and support based on patient preference

### 24/7 TROUBLESHOOTING SUPPORT

# Prepared to Meet the Needs of PNH Patients

#### VALUE & ACCESS

- Over 50 unique payer interactions completed
- Identified and engaging with high priority payers representing >80% of all U.S. PNH patients
- Distribution model and patient support resources activated

#### MARKETING

PNH strategy defined



Disease education ongoing

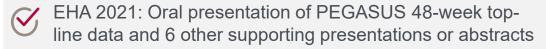


Digital marketing performing well above industry benchmarks



#### MEDICAL AFFAIRS





Integration of patient perspective into education and patient support resources

### SALES

Salesforce deployed March 1



Customer segmentation and targeting complete

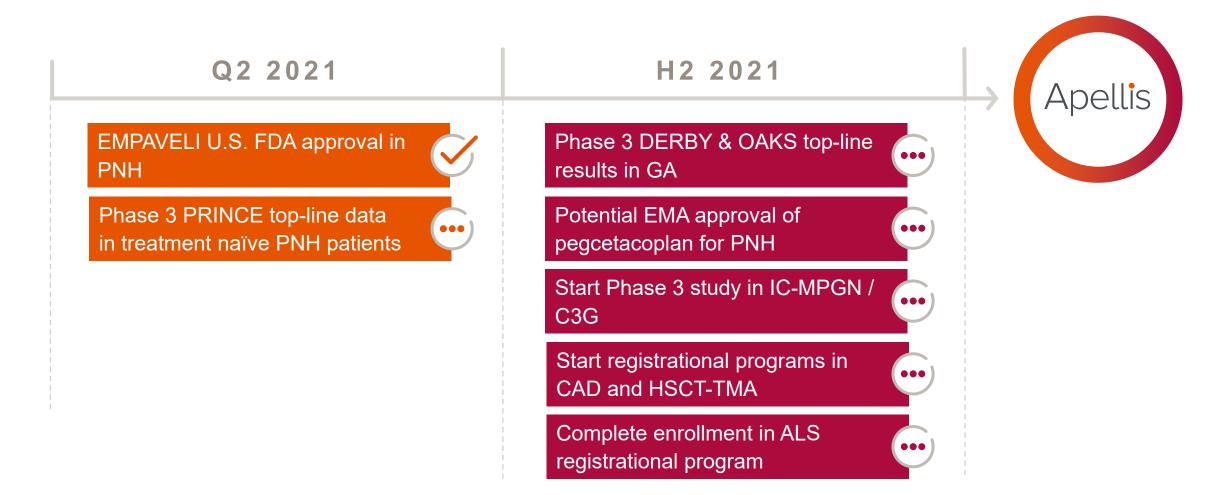


Virtual engagements informing strategic account planning





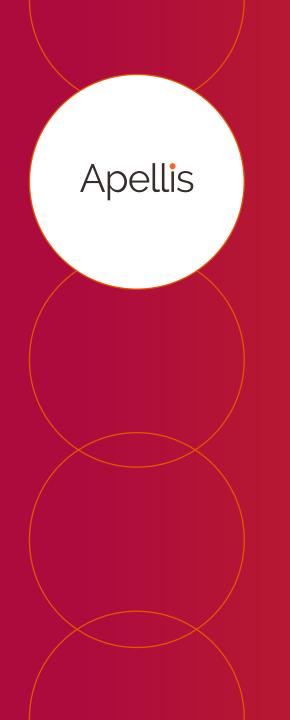
### 2021: Transformational Year



### **Our Sincere Thanks**

to employees, patients, caregivers, investigators & investors for their support





Q&A



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