



# Topline Results of Phase II AURORA Trial of Avacopan for Hidradenitis Suppurativa (HS)

October 28, 2020

# Forward-Looking Statements

This presentation contains forward-looking statements that involve substantial risks and uncertainties. In some cases, you can identify forward-looking statements by the following words: "may," "will," "could," "would," "should," "expect," "intend," "plan," "anticipate," "contemplate," "believe," "estimate," "predict," "project," "seek," "potential," "continue," "ongoing" or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. These statements relate to future events or our future financial performance or condition and involve known and unknown risks, uncertainties and other factors that could cause our actual results, levels of activity, performance or achievement to differ materially from those expressed or implied by these forward-looking statements. These risks, uncertainties and other factors are described more fully in our periodic reports filed with the Securities and Exchange Commission (SEC), including our Annual Report on Form 10-K for the year ended December 31, 2019, filed with the SEC on March 10, 2020, and our Form 10-Q filed on August 10, 2020, particularly in the sections titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations". In light of the significant uncertainties in our forward-looking statements, you should not place undue reliance on these statements or regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified timeframe, or at all. The forward-looking statements contained in this presentation represent our estimates and assumptions only as of the date of this presentation and, except as required by law, we undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise after the date of this presentation.

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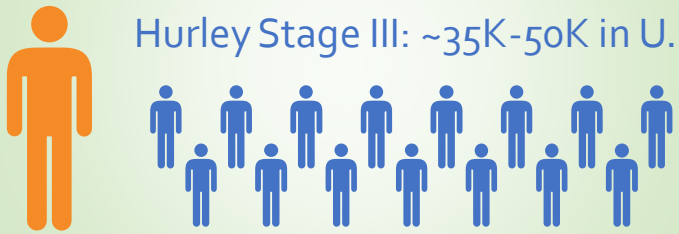
# Phase II AURORA Trial Topline Results Summary

- Avacopan demonstrated statistically significant dose-dependent improvement in HiSCR (Hidradenitis Suppurativa Clinical Response) vs. placebo in pre-specified Hurley Stage III (severe HS) patients at 12 weeks
- Numerical improvement in HiSCR observed with avacopan treatment in overall study population (Hurley Stage II & III) though did not reach statistical significance
- Consistent reduction in Hurley Stage III patients in International HS Severity Score (IHS4), as well as reduction in AN (abscesses and inflammatory nodules), draining fistula, and abscess count at week 12
- Avacopan shown to be safe and well tolerated
- Company plans to pursue Phase III development for avacopan in Hurley Stage III (severe HS) patients; sizable unmet need and potential market opportunity

# Hidradenitis Suppurativa (HS): A Debilitating Skin Disease

**HS is a chronic, disabling autoimmune skin disease featuring painful, disfiguring nodules, boils and abscesses**

**~200K patients with moderate (Hurley Stage II) to severe (Hurley Stage III) HS in the U.S. alone**




## Current Treatments

- Adalimumab is the only approved drug for HS; widely regarded as only having moderate efficacy
- Still, sales in adalimumab in HS >$1B
- Precedent for FDA Orphan Drug Designation for moderate or severe HS

## Rationale for Avacopan

- HS thought to be a neutrophil-driven disease where C5a involvement is validated (C5aR is elevated in skin biopsies of HS patients[1])
- C5a blockade with avacopan via C5aR offers a strong potential to control neutrophil activation
- Oral application offers advantages over injections (e.g. adalimumab) or infusions

**Significant need for new treatment options for HS**

[1]Hoffman et al. PLoS One. 2018;13(9):e0203672



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# Phase II AURORA Study Design


## Trial Design:

- Double-blind, placebo-controlled Phase II trial
- Randomized 398 patients randomized (1:1:1) across ~100 sites
- Enrolled Hurley Stage II (moderate) or III (severe)
- Placebo crosses over to active at 12 weeks, follow all groups for additional 24 weeks

## Primary Endpoint:

- Proportion of patients with clinical response (HiSCR) at 12 weeks

## Secondary Endpoints Include:

- Reduction in International HS Severity Score ($IHS_4$) from baseline to Week 12
- Proportion of patients with baseline who achieve abscess and inflammatory nodule reduction baseline to Week 12
- Proportion of patients achieving reduction and baseline to Week 12 in global assessment of skin pain

# Patient Disposition & Baseline Characteristics

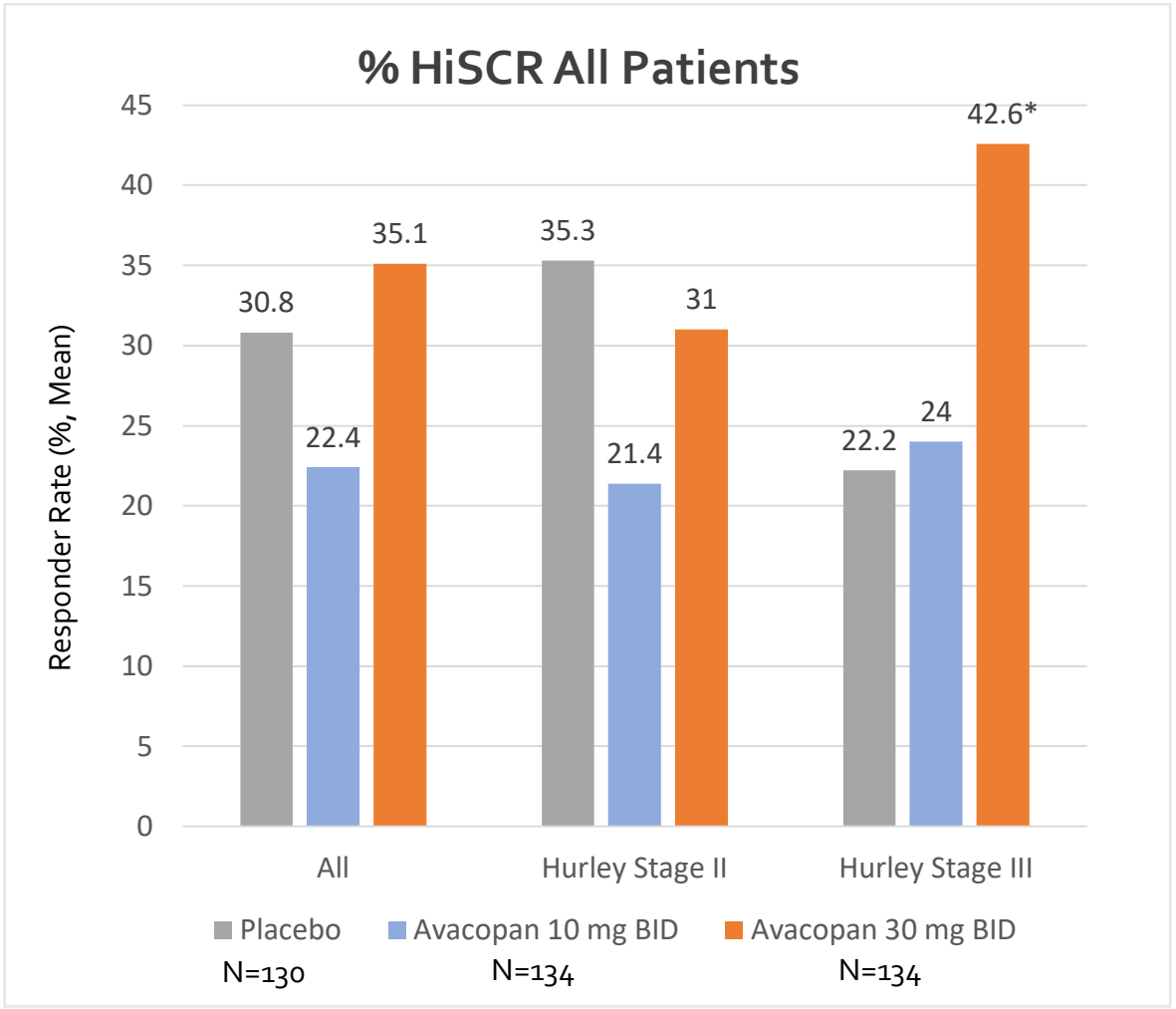
## Patient Disposition

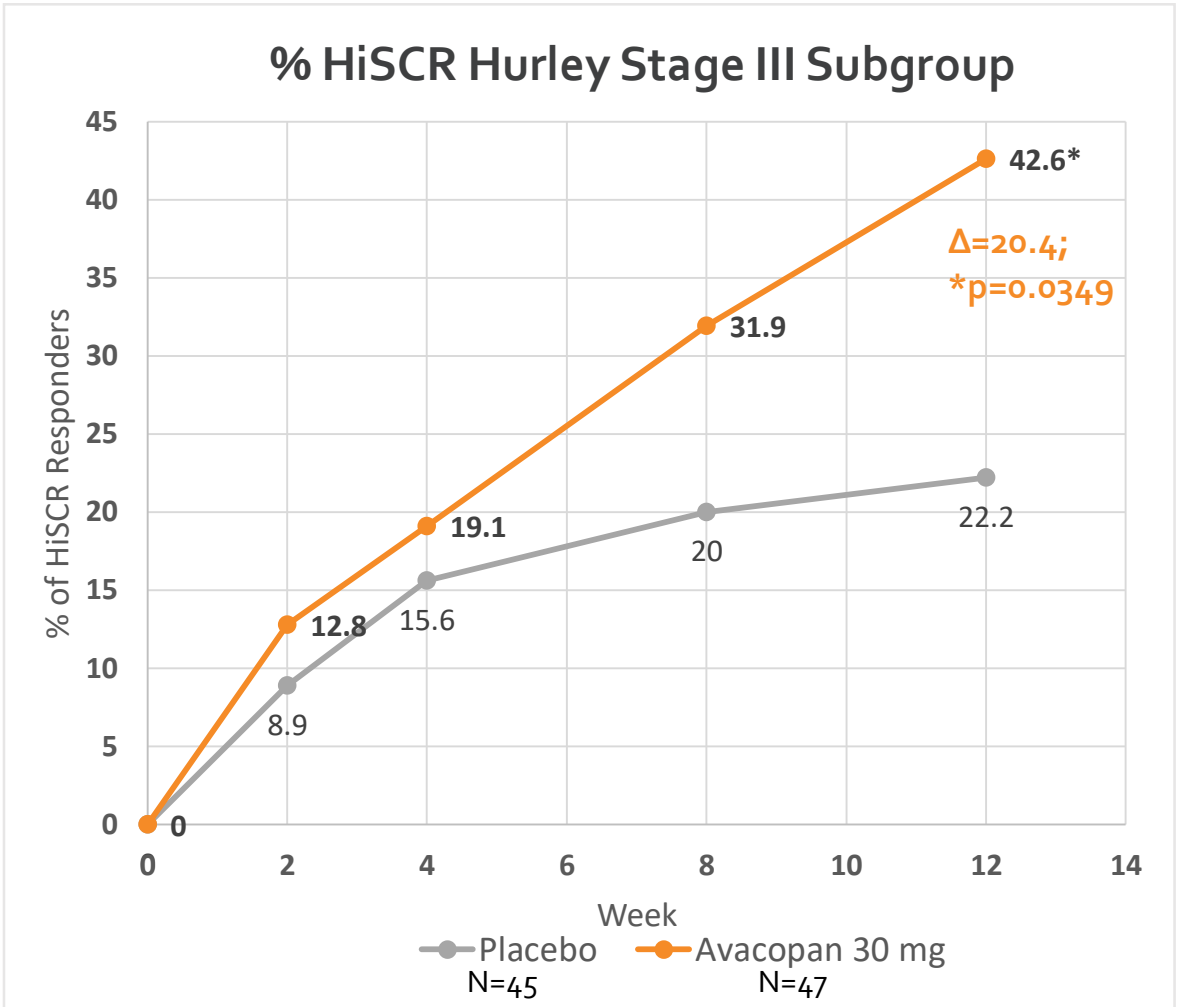
| | Placebo | Avacopan 10 mg BID | Avacopan 30 mg BID | Avacopan Total |
|---|---|---|---|---|
| **ITT (N)** | **130** | **134** | **134** | **268** |
| Completed week 12 (N) | 118 | 115 | 118 | 233 |
| Withdrew Early From Study (N) | 25 | 30 | 29 | 59 |

## ITT Population: Baseline Characteristics

| | | Placebo (N=130) | Avacopan 10 mg BID (N=134) | Avacopan 30 mg BID (N=134) | Avacopan Total (N=268) |
|---|---|---|---|---|---|
| **Age** | Years (Mean) | 36.9 (11.87) | 36.0 (12.05) | 36.8 (11.57) | 36.4 (11.80) |
| **Sex** | Female (%) | 82.3 | 77.6 | 79.1 | 78.4 |
| **Race** | White (%) | 63.8 | 61.2 | 71.6 | 66.4 |
| | Black or African-American (%) | 33.1 | 34.3 | 25.4 | 29.9 |
| **BMI** | $kg/m^2$ (Mean) | 36.87 | 36.14 | 36.75 | 36.44 |
| **HS Disease Duration** | Years (Mean) | 10.99 | 10.64 | 11.21 | 10.93 |
| **Hurley Stage** | Stage II (%) | 65.4 | 62.7 | 64.9 | 63.8 |
| | Stage III (%) | 34.6 | 37.3 | 35.1 | 36.2 |
| **Previous TNF-alpha Inhibitor Use** | (%) | 26.9 | 28.4 | 26.9 | 27.6 |

# Primary Endpoint: Proportion of Patients with Clinical Response (HiSCR) at 12 Weeks




*p=0.0349



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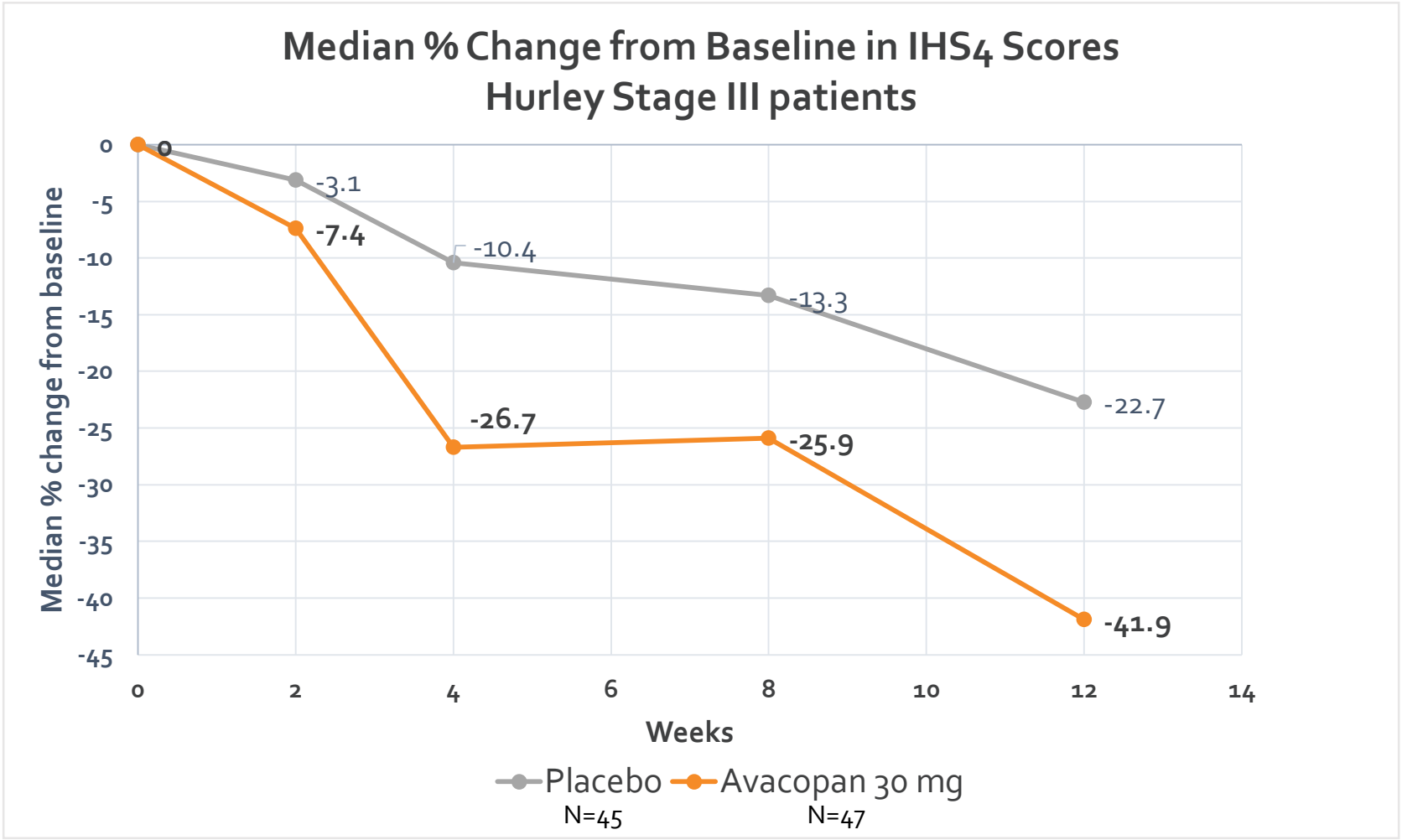
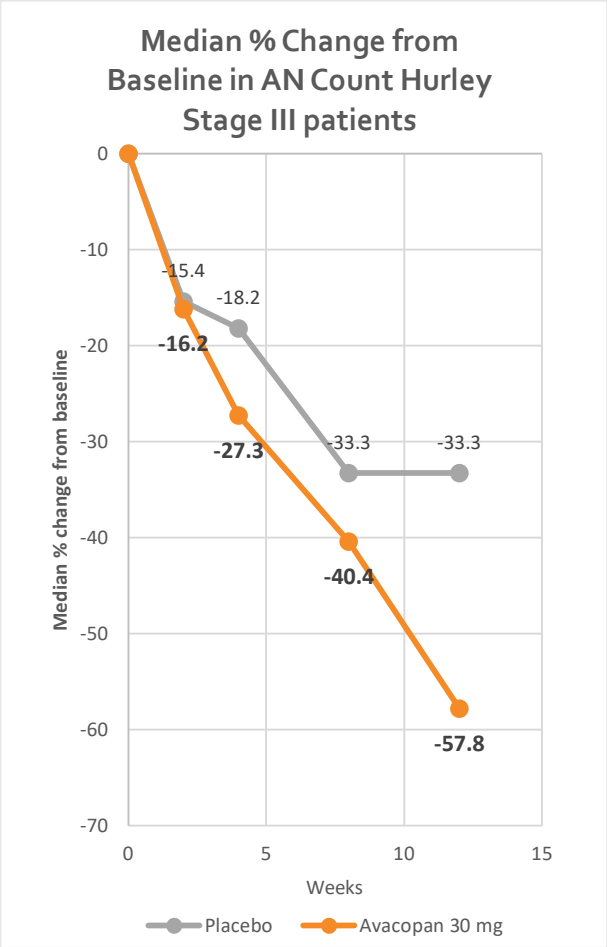
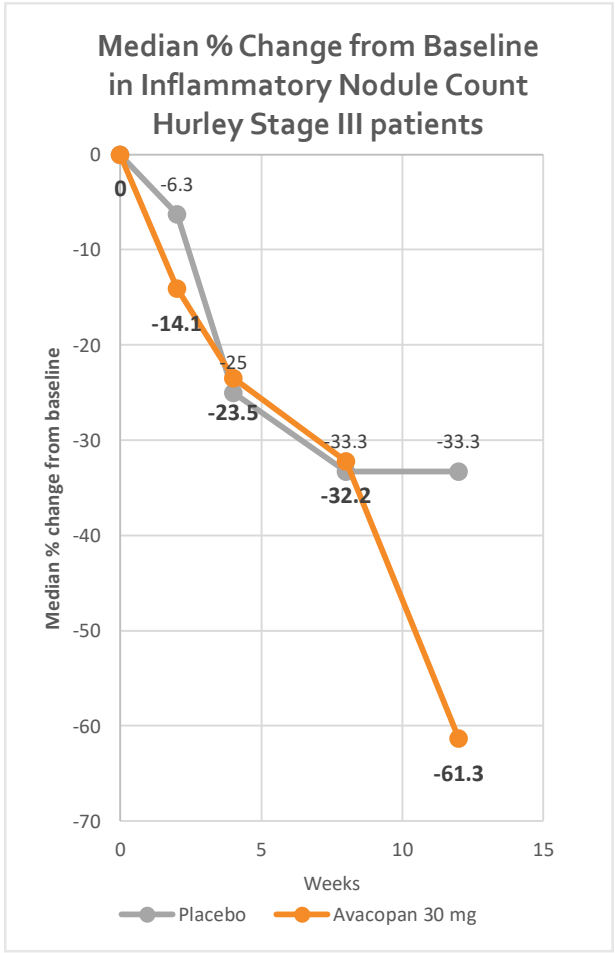
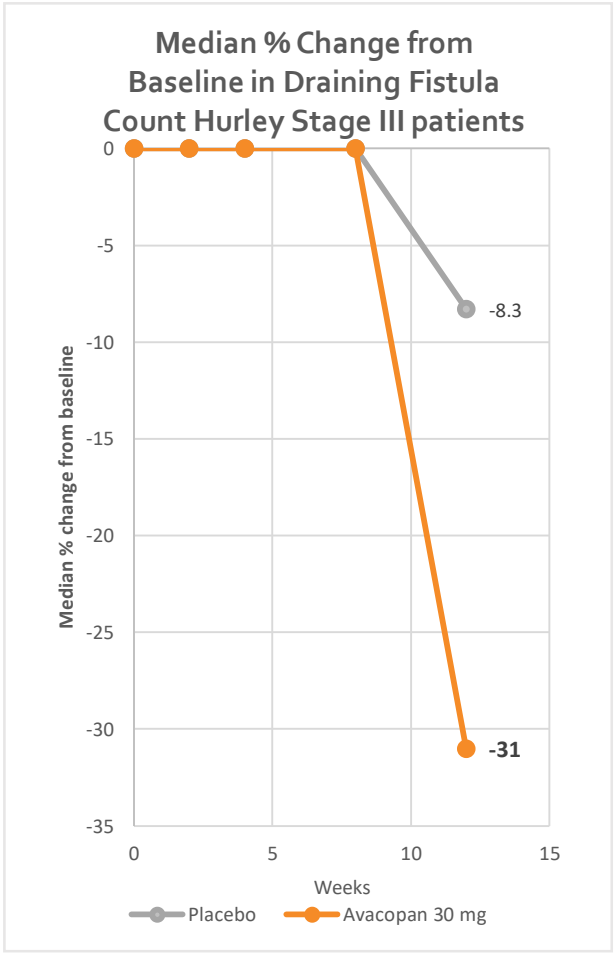
# Change in IHS4 Scores in Hurley Stage III Responders

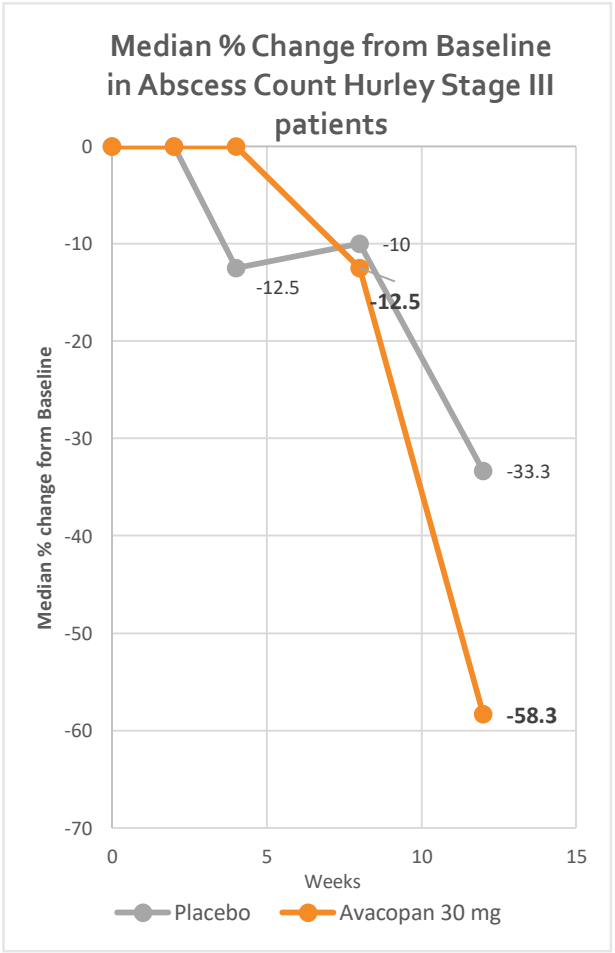


# Observations in Lesion Counts Support % HiSCR Hurley Stage III Responders




Placebo N=45; Avacopan 30mg N=47



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# Avacopan Demonstrated a Favorable Safety Profile and Was Well-Tolerated in HS Patients

- Treatment emergent adverse events (TEAEs) of all types were observed to be fewer in the avacopan groups (48.5%) than with placebo (55%).
- The most frequent TEAEs reported across study groups (≥5% in any group) were hidradenitis, headache, and nausea with the majority being mild to moderate.

| | Placebo (n=129) | Avacopan 10mg (n=134) | Avacopan 30 mg (n=135) | Avacopan total (n=269) |
|---|---|---|---|---|
| **TEAEs** | 71 (55.0%) | 64 (47.8%) | 66 (48.9%) | 130 (48.3%) |
| Mild | 35 (27.1%) | 32 (23.9%) | 26 (19.3%) | 58 (21.6%) |
| Moderate | 27 (20.9%) | 20 (14.9%) | 33 (24.4%) | 53 (19.7%) |
| Severe | 9 (7.0%) | 12 (9%) | 7 (5.2%) | 19 (7.1%) |
| **Serious TEAEs** | 3 (2.3%) | 3 (2.2%) | 1 (0.7%) | 4 (1.5%) |

# Next Steps: Avacopan in 2020-2021

- Phase II AURORA trial: Hurley Stage III (severe HS patients) identified as the target patient population for avacopan treatment
- Company plans to pursue Phase III development for avacopan in Hurley Stage III patients; sizable potential market opportunity with ~35-50K patients in the U.S. alone
- Avacopan continues to advance for other indications:
  - U.S. NDA under review for avacopan in ANCA vasculitis with a PDUFA date of July 7, 2021; EMA validation expected by year-end and JNDA to follow
  - Continue to prepare for potential commercialization in the U.S.
  - Topline data from ACCOLADE trial in C3G expected by YE 2020
  - Explore and plan for additional renal indications such as Lupus Nephritis (LN)