

Dataset Integrity Check for the  
Extension Study to Evaluate the Long-  
Term Safety and Durability of Effect of  
LUM001 in the Treatment of Cholestatic  
Liver Disease in Pediatric Subjects With  
Alagille Syndrome (IMAGINE-II)

Prepared by NIDDK-CR  
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## 1 Standard Disclaimer

The intent of this DSIC is to provide confidence that the data distributed by the NIDDK repository is a true copy of the study data. Our intent is not to assess the integrity of the statistical analyses reported by study investigators. As with all statistical analyses of complex datasets, complete replication of a set of statistical results should not be expected in secondary analysis. This occurs for a number of reasons including differences in the handling of missing data, restrictions on cases included in samples for a particular analysis, software coding used to define complex variables, etc. Experience suggests that most discrepancies can ordinarily be resolved by consultation with the study data coordinating center (DCC), however this process is labor-intensive for both DCC and Repository staff. It is thus not our policy to resolve every discrepancy that is observed in an integrity check. Specifically, we do not attempt to resolve minor or inconsequential discrepancies with published results or discrepancies that involve complex analyses, unless NIDDK Repository staff suspect that the observed discrepancy suggests that the dataset may have been corrupted in storage, transmission, or processing by repository staff. We do, however, document in footnotes to the integrity check those instances in which our secondary analyses produced results that were not fully consistent with those reported in the target publication.

## 2 Study Background

The IMAGINE-II study was a multicenter, extension study of LUM001 in children diagnosed with Alagille syndrome (ALGS) who had completed participation in a core LUM001 treatment protocol (ITCH study). The primary objective was to evaluate long-term safety and tolerability of LUM001. Efficacy was assessed by evaluating the effect of LUM001 on the biochemical markers and pruritus associated with ALGS.

## 3 Archived Datasets

A full listing of archived datasets included in the package can be found in the Roadmap document. All data files, as provided by the Data Coordinating Center (DCC), are located in the IMAGINE-II folder in the data package. For this replication, variables were taken from the “dm.sas7bdat” dataset.

## 4 Statistical Methods

Analyses were performed to replicate results posted to ClinicalTrials.gov [1]. To verify the integrity of the data, only descriptive statistics were computed.

## 5 Results

For the table on ClinicalTrials.gov [1], Baseline Characteristics, Table A lists the variables that were used in the replication, and Table B compares the results calculated from the archived data files to the results in the table. The results of the replication are within expected variation to the published results.

## 6 Conclusions

The NIDDK Central Repository is confident that the IMAGINE-II data files to be distributed are a true copy of the study data.

## 7 References

[1] An Extension Study to Evaluate the Long-Term Safety and Durability of Effect of LUM001 in the Treatment of Cholestatic Liver Disease in Pediatric Subjects With Alagille Syndrome (IMAGINE-II). ClinicalTrials.gov, July 2021, <https://clinicaltrials.gov/study/NCT02117713>

**Table A:** Variables used to replicate the website table – Baseline Characteristics

<b>Table Variable</b>	<b>dataset.variable</b>
Age	dm.age
Sex	dm.sex
Race	dm.race
Region of Enrollment	dm.country

**Table B:** Comparison of values computed in integrity check to reference website table

<b>Baseline Characteristics</b>	<b>ClinicalTrials.gov Results (n=34)</b>	<b>DSIC (n=34)</b>	<b>Diff. (n=0)</b>
Age, Mean (Standard Deviation)	7 (4.55)	7 (4.54)	0 (0.01)
Sex, n (%)			
Female	14 (41.2)	14 (41.2)	0 (0)
Male	20 (58.8)	20 (58.8)	0 (0)
Race, n (%)			
American Indian or Alaska Native	0 (0.0)	0 (0.0)	0 (0)
Asian	1 (2.9)	1 (2.9)	0 (0)
Native Hawaiian or Other Pacific Islander	0 (0.0)	0 (0.0)	0 (0)
Black or African American	4 (11.8)	4 (11.8)	0 (0)
White	27 (79.4)	27 (79.4)	0 (0)
More than one race	1 (2.9)	1 (2.9)	0 (0)
Unknown or Not Reported	1 (2.9)	1 (2.9)	0 (0)
Region of Enrollment, n			
Canada	4	4	0
United States	30	30	0

## Attachment A: SAS Code

```
libname imagii "X:\NIDDK\niddk-dr_studies2\IMAGINE-II\private_created_data\Data\Redacted  
Data\IM2_SDTM_Archival_SAS_ADaM_datasets";  
libname imagii2 "X:\NIDDK\niddk-dr_studies2\IMAGINE-II\private_created_data\Data\Redacted  
Data\IM2_SDTM_Archival_SAS_SDTM_datasets";
```

```
/******  
/* IMAGINE-II DSIC */  
/******
```

```
*demographics dataset;  
*dm;  
data dm; set imagii2.dm;  
run;
```

```
*age;  
proc means data=dm maxdec=2;  
var age;  
run;
```

```
*sex;  
proc freq data=dm;  
tables sex;  
run;
```

```
*race;  
proc freq data=dm;  
tables race/missing;  
run;
```

```
*country;  
proc freq data=dm;  
tables country;  
run;
```