

LUM001305 Study

Blank CRF Casebook

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Protocol: **LUM001-305** Study Version: **8.3**

Time & Events Schedule

Legend: Expected Expected, Restricted Optional Optional, Restricted

Name	Assessment	Day 0	Week 1	Week 2	Week 3	Week 4	Week 6	Week 8	Week 10	Week 12	Week 16
DM	Informed Consent/Demography										
IE1	Inclusion/Exclusion Criteria										
MH	Medical History										
TC	Telephone Contact										
VS	Vital Signs										
LB2	Clinical Laboratory Tests 2										
QS1	Scratch Scale										
PREG2	Urine Pregnancy Test										
EX	Study Drug Administration										
SDC	Study Drug Compliance										
DAD	Dose Adjustment										
PE2	Physical Examination2										
LB3	Clinical Laboratory Tests 3										
QS2	Questionnaires 2										
QS3P	Pediatric QOL Inventory PARENT RPT-INFANT 1-12M										
QS3Q	Pediatric QOL Inventory PARENT RPT-INFANT 13-24M										
QS3A	Pediatric QOL Inventory PARENT RPT (TODDLERS)										

Name	Assessment	Day 0	Week 1	Week 2	Week 3	Week 4	Week 6	Week 8	Week 10	Week 12	Week 16
QS3B	Pediatric QOL Inventory PARENT RPT (YOUNG CHILD)										
QS3C	Pediatric QOL Inventory PARENT RPT (CHILDREN)										
QS3D	Pediatric QOL Inventory PARENT RPT (TEENS)										
QS3E	Pediatric QOL Inventory YOUNG CHILD REPORT										
QS3F	Pediatric QOL Inventory CHILD REPORT										
QS3G	Pediatric QOL Inventory TEEN REPORT										
QS3H	Multidimensional Fatigue Scale PARENT for TODDLERS										
QS3I	Multidimensional Fatigue Scale PARENT (YOUNG CHILD)										
QS3J	Multidimensional Fatigue Scale PARENT for CHILDREN										
QS3K	Multidimensional Fatigue Scale PARENT for TEENS										

Name	Assessment	Day 0	Week 1	Week 2	Week 3	Week 4	Week 6	Week 8	Week 10	Week 12	Week 16
QS3L	Multidimensional Fatigue Scale YOUNG CHILD										
QS3M	Multidimensional Fatigue Scale CHILD										
QS3N	Multidimensional Fatigue Scale TEEN										
QS3O	Family Impact Module PARENT REPORT										
QS7	Xanthoma Scale										
QS5	Caregiver Impression of Change										
DS	Subject Summary										
PRE	Protocol Extension										
IE2	Inclusion/Exclusion Criteria 2										
MH2	Medical History 2										
LB	Clinical Laboratory Tests										
PRE2	Long Term Optional Treatment										
IE3	Inclusion/Exclusion Criteria 3										
DS2	Subject Summary 2										
PRE3	Long Term Optional Treatment 2										
CM	Concomitant Medications										
EX2	Study Drug Administration 2										

Name	Assessment	Day 0	Week 1	Week 2	Week 3	Week 4	Week 6	Week 8	Week 10	Week 12	Week 16
AE	Adverse Events										
SDA1	Study Drug Accountability 1										
Scr	Screened										
Enroll	Enrolled										
Fail	Failed										
Disc	Discontinued										
Comp	Completed										





















Name	Assessment	Week 20	Week 24	Week 28	Week 32	Week 36	Week 40	Week 44	Week 48	Post-Week 48 Follow-up	Study Completion (Complete)
DM	Informed Consent/Demography										
IE1	Inclusion/Exclusion Criteria										
MH	Medical History										
TC	Telephone Contact	✓		✓	✓		✓			✓	
VS	Vital Signs		✓			✓			✓		
LB2	Clinical Laboratory Tests 2		✓			✓			✓		
QS1	Scratch Scale		✓			✓			✓		
PREG2	Urine Pregnancy Test		✓			✓			✓		
EX	Study Drug Administration										
SDC	Study Drug Compliance		✓			✓		✓	✓		
DAD	Dose Adjustment										
PE2	Physical Examination2		✓			✓			✓		
LB3	Clinical Laboratory Tests 3										
QS2	Questionnaires 2		✓						✓		
QS3P	Pediatric QOL Inventory PARENT RPT-INFANT 1-12M		✓						✓		
QS3Q	Pediatric QOL Inventory PARENT RPT-INFANT 13-24M		✓						✓		
QS3A	Pediatric QOL Inventory PARENT RPT (TODDLERS)		✓						✓		

Name	Assessment	Week 20	Week 24	Week 28	Week 32	Week 36	Week 40	Week 44	Week 48	Post-Week 48 Follow-up	Study Completion (Complete)
QS3B	Pediatric QOL Inventory PARENT RPT (YOUNG CHILD)										
QS3C	Pediatric QOL Inventory PARENT RPT (CHILDREN)										
QS3D	Pediatric QOL Inventory PARENT RPT (TEENS)										
QS3E	Pediatric QOL Inventory YOUNG CHILD REPORT										
QS3F	Pediatric QOL Inventory CHILD REPORT										
QS3G	Pediatric QOL Inventory TEEN REPORT										
QS3H	Multidimensional Fatigue Scale PARENT for TODDLERS										
QS3I	Multidimensional Fatigue Scale PARENT (YOUNG CHILD)										
QS3J	Multidimensional Fatigue Scale PARENT for CHILDREN										
QS3K	Multidimensional Fatigue Scale PARENT for TEENS										

Name	Assessment	Week 20	Week 24	Week 28	Week 32	Week 36	Week 40	Week 44	Week 48	Post-Week 48 Follow-up	Study Completion (Complete)
QS3L	Multidimensional Fatigue Scale YOUNG CHILD										
QS3M	Multidimensional Fatigue Scale CHILD										
QS3N	Multidimensional Fatigue Scale TEEN										
QS3O	Family Impact Module PARENT REPORT										
QS7	Xanthoma Scale										
QS5	Caregiver Impression of Change										
DS	Subject Summary										
PRE	Protocol Extension										
IE2	Inclusion/Exclusion Criteria 2										
MH2	Medical History 2										
LB	Clinical Laboratory Tests										
PRE2	Long Term Optional Treatment										
IE3	Inclusion/Exclusion Criteria 3										
DS2	Subject Summary 2										
PRE3	Long Term Optional Treatment 2										
CM	Concomitant Medications										
EX2	Study Drug Administration 2										

Name	Assessment	Week 20	Week 24	Week 28	Week 32	Week 36	Week 40	Week 44	Week 48	Post-Week 48 Follow-up	Study Completion (Complete)
AE	Adverse Events										
SDA1	Study Drug Accountability 1										
Scr	Screened										
Enroll	Enrolled										
Fail	Failed										
Disc	Discontinued										
Comp	Completed										

Name	Assessment	Study Completion (Discontinue)	Re-Initiation Day 0	Re-Initiation Week 49	Re-Initiation Week 50	Re-Initiation Week 51	Re-Initiation Week 52	Week 56	Week 60	Week 64
DM	Informed Consent/Demography									
IE1	Inclusion/Exclusion Criteria									
MH	Medical History									
TC	Telephone Contact			✓		✓	✓	✓		✓
VS	Vital Signs		✓		✓		✓		✓	
LB2	Clinical Laboratory Tests 2				✓		✓			
QS1	Scratch Scale		✓		✓		✓		✓	
PREG2	Urine Pregnancy Test		✓		✓		✓		✓	
EX	Study Drug Administration									
SDC	Study Drug Compliance				✓		✓		✓	
DAD	Dose Adjustment									
PE2	Physical Examination2		✓						✓	
LB3	Clinical Laboratory Tests 3									
QS2	Questionnaires 2		✓						✓	
QS3P	Pediatric QOL Inventory PARENT RPT-INFANT 1-12M		✓						✓	
QS3Q	Pediatric QOL Inventory PARENT RPT-INFANT 13-24M		✓						✓	
QS3A	Pediatric QOL Inventory PARENT RPT (TODDLERS)		✓						✓	

Name	Assessment	Study Completion (Discontinue)	Re-Initiation Day 0	Re-Initiation Week 49	Re-Initiation Week 50	Re-Initiation Week 51	Re-Initiation Week 52	Week 52	Week 56	Week 60	Week 64
QS3B	Pediatric QOL Inventory PARENT RPT (YOUNG CHILD)										
QS3C	Pediatric QOL Inventory PARENT RPT (CHILDREN)										
QS3D	Pediatric QOL Inventory PARENT RPT (TEENS)										
QS3E	Pediatric QOL Inventory YOUNG CHILD REPORT										
QS3F	Pediatric QOL Inventory CHILD REPORT										
QS3G	Pediatric QOL Inventory TEEN REPORT										
QS3H	Multidimensional Fatigue Scale PARENT for TODDLERS										
QS3I	Multidimensional Fatigue Scale PARENT (YOUNG CHILD)										
QS3J	Multidimensional Fatigue Scale PARENT for CHILDREN										
QS3K	Multidimensional Fatigue Scale PARENT for TEENS										

Name	Assessment	Study Completion (Discontinue)	Re-Initiation Day 0	Re-Initiation Week 49	Re-Initiation Week 50	Re-Initiation Week 51	Re-Initiation Week 52	Week 52	Week 56	Week 60	Week 64
QS3L	Multidimensional Fatigue Scale YOUNG CHILD										
QS3M	Multidimensional Fatigue Scale CHILD										
QS3N	Multidimensional Fatigue Scale TEEN										
QS3O	Family Impact Module PARENT REPORT										
QS7	Xanthoma Scale										
QS5	Caregiver Impression of Change										
DS	Subject Summary										
PRE	Protocol Extension										
IE2	Inclusion/Exclusion Criteria 2										
MH2	Medical History 2										
LB	Clinical Laboratory Tests										
PRE2	Long Term Optional Treatment										
IE3	Inclusion/Exclusion Criteria 3										
DS2	Subject Summary 2										
PRE3	Long Term Optional Treatment 2										
CM	Concomitant Medications										
EX2	Study Drug Administration 2										

Name	Assessment	Study Completion (Discontinue)	Re-Initiation Day 0	Re-Initiation Week 49	Re-Initiation Week 50	Re-Initiation Week 51	Re-Initiation Week 52	Week 52	Week 56	Week 60	Week 64
AE	Adverse Events										
SDA1	Study Drug Accountability 1										
Scr	Screened										
Enroll	Enrolled										
Fail	Failed										
Disc	Discontinued										
Comp	Completed										





















Name	Assessment	Week 68	Week 72	Week 76	Week 80	Week 84	Week 88	Week 92	Week 96_ET	30 Day Follow-up	Extension Completion
DM	Informed Consent/Demography										
IE1	Inclusion/Exclusion Criteria										
MH	Medical History										
TC	Telephone Contact	✓		✓	✓	✓	✓	✓		✓	
VS	Vital Signs		✓			✓			✓		
LB2	Clinical Laboratory Tests 2										
QS1	Scratch Scale		✓			✓			✓		
PREG2	Urine Pregnancy Test		✓			✓			✓		
EX	Study Drug Administration										
SDC	Study Drug Compliance		✓			✓			✓		
DAD	Dose Adjustment										
PE2	Physical Examination2		✓			✓			✓		
LB3	Clinical Laboratory Tests 3										
QS2	Questionnaires 2		✓			✓			✓		
QS3P	Pediatric QOL Inventory PARENT RPT-INFANT 1-12M		✓			✓			✓		
QS3Q	Pediatric QOL Inventory PARENT RPT-INFANT 13-24M		✓			✓			✓		
QS3A	Pediatric QOL Inventory PARENT RPT (TODDLERS)		✓			✓			✓		

Name	Assessment	Week 68	Week 72	Week 76	Week 80	Week 84	Week 88	Week 92	Week 96_ET	30 Day Follow-up	Extension Completion
QS3B	Pediatric QOL Inventory PARENT RPT (YOUNG CHILD)	✓	✓			✓			✓		
QS3C	Pediatric QOL Inventory PARENT RPT (CHILDREN)		✓			✓			✓		
QS3D	Pediatric QOL Inventory PARENT RPT (TEENS)		✓			✓			✓		
QS3E	Pediatric QOL Inventory YOUNG CHILD REPORT		✓			✓			✓		
QS3F	Pediatric QOL Inventory CHILD REPORT		✓			✓			✓		
QS3G	Pediatric QOL Inventory TEEN REPORT		✓			✓			✓		
QS3H	Multidimensional Fatigue Scale PARENT for TODDLERS		✓			✓			✓		
QS3I	Multidimensional Fatigue Scale PARENT (YOUNG CHILD)		✓			✓			✓		
QS3J	Multidimensional Fatigue Scale PARENT for CHILDREN		✓			✓			✓		
QS3K	Multidimensional Fatigue Scale PARENT for TEENS		✓			✓			✓		

Name	Assessment	Week 68	Week 72	Week 76	Week 80	Week 84	Week 88	Week 92	Week 96_ET	30 Day Follow-up	Extension Completion
QS3L	Multidimensional Fatigue Scale YOUNG CHILD		✔			✔			✔		
QS3M	Multidimensional Fatigue Scale CHILD		✔			✔			✔		
QS3N	Multidimensional Fatigue Scale TEEN		✔			✔			✔		
QS3O	Family Impact Module PARENT REPORT		✔			✔			✔		
QS7	Xanthoma Scale		✔			✔			✔		
QS5	Caregiver Impression of Change		✔			✔			✔		
DS	Subject Summary										✔
PRE	Protocol Extension										
IE2	Inclusion/Exclusion Criteria 2										
MH2	Medical History 2										
LB	Clinical Laboratory Tests		✔			✔			✔		
PRE2	Long Term Optional Treatment										
IE3	Inclusion/Exclusion Criteria 3										
DS2	Subject Summary 2										
PRE3	Long Term Optional Treatment 2										
CM	Concomitant Medications										
EX2	Study Drug Administration 2										

Name	Assessment	Week 68	Week 72	Week 76	Week 80	Week 84	Week 88	Week 92	Week 96_ET	30 Day Follow-up	Extension Completion
AE	Adverse Events										
SDA1	Study Drug Accountability 1										
Scr	Screened										
Enroll	Enrolled										
Fail	Failed										
Disc	Discontinued										
Comp	Completed										

Name	Assessment	Long Term Optional Treatment	PA5 Re-Initiation Day -2	PA5 Re-Initiation Day 0	PA5 Re-Initiation Week 1	PA5 Re-Initiation Week 2	PA5 Re-Initiation Week 3	PA5 Re-Initiation Week 4	Week 100	Week 104	Week 108
DM	Informed Consent/Demography										
IE1	Inclusion/Exclusion Criteria										
MH	Medical History										
TC	Telephone Contact				✓		✓		✓	✓	
VS	Vital Signs		✓	✓		✓		✓			✓
LB2	Clinical Laboratory Tests 2										
QS1	Scratch Scale			✓		✓		✓			✓
PREG2	Urine Pregnancy Test			✓		✓		✓			✓
EX	Study Drug Administration										
SDC	Study Drug Compliance					✓		✓			✓
DAD	Dose Adjustment										
PE2	Physical Examination2		✓	✓		✓		✓			✓
LB3	Clinical Laboratory Tests 3			✓		✓		✓			✓
QS2	Questionnaires 2			✓							✓
QS3P	Pediatric QOL Inventory PARENT RPT-INFANT 1-12M			✓							✓
QS3Q	Pediatric QOL Inventory PARENT RPT-INFANT 13-24M			✓							✓
QS3A	Pediatric QOL Inventory PARENT RPT (TODDLERS)			✓							✓

Name	Assessment	Long Term Optional Treatment	PA5 Re- Initiation Day -2	PA5 Re- Initiation Day 0	PA5 Re- Initiation Week 1	PA5 Re- Initiation Week 2	PA5 Re- Initiation Week 3	PA5 Re- Initiation Week 4	Week 100	Week 104	Week 108
QS3B	Pediatric QOL Inventory PARENT RPT (YOUNG CHILD)										
QS3C	Pediatric QOL Inventory PARENT RPT (CHILDREN)										
QS3D	Pediatric QOL Inventory PARENT RPT (TEENS)										
QS3E	Pediatric QOL Inventory YOUNG CHILD REPORT										
QS3F	Pediatric QOL Inventory CHILD REPORT										
QS3G	Pediatric QOL Inventory TEEN REPORT										
QS3H	Multidimensional Fatigue Scale PARENT for TODDLERS										
QS3I	Multidimensional Fatigue Scale PARENT (YOUNG CHILD)										
QS3J	Multidimensional Fatigue Scale PARENT for CHILDREN										
QS3K	Multidimensional Fatigue Scale PARENT for TEENS										

Name	Assessment	Long Term Optional Treatment	PA5 Re-Initiation Day - 2	PA5 Re-Initiation Day 0	PA5 Re-Initiation Week 1	PA5 Re-Initiation Week 2	PA5 Re-Initiation Week 3	PA5 Re-Initiation Week 4	Week 100	Week 104	Week 108
QS3L	Multidimensional Fatigue Scale YOUNG CHILD										
QS3M	Multidimensional Fatigue Scale CHILD										
QS3N	Multidimensional Fatigue Scale TEEN										
QS3O	Family Impact Module PARENT REPORT										
QS7	Xanthoma Scale										
QS5	Caregiver Impression of Change										
DS	Subject Summary										
PRE	Protocol Extension										
IE2	Inclusion/Exclusion Criteria 2										
MH2	Medical History 2										
LB	Clinical Laboratory Tests										
PRE2	Long Term Optional Treatment										
IE3	Inclusion/Exclusion Criteria 3										
DS2	Subject Summary 2										
PRE3	Long Term Optional Treatment 2										
CM	Concomitant Medications										
EX2	Study Drug Administration 2										

Name	Assessment	Long Term Optional Treatment	PA5 Re- Initiation Day -2	PA5 Re- Initiation Day 0	PA5 Re- Initiation Week 1	PA5 Re- Initiation Week 2	PA5 Re- Initiation Week 3	PA5 Re- Initiation Week 4	Week 100	Week 104	Week 108
AE	Adverse Events										
SDA1	Study Drug Accountability 1										
Scr	Screened										
Enroll	Enrolled										
Fail	Failed										
Disc	Discontinued										
Comp	Completed										

Name	Assessment	Week 112	Week 116	Week 120	Week 124	Week 128	Week 132	Week 136	Week 140	Week 144_ET	Week 148
DM	Informed Consent/Demography										
IE1	Inclusion/Exclusion Criteria										
MH	Medical History										
TC	Telephone Contact	✓	✓		✓	✓	✓	✓			✓
VS	Vital Signs			✓			✓			✓	✓
LB2	Clinical Laboratory Tests 2									✓	✓
QS1	Scratch Scale			✓			✓			✓	✓
PREG2	Urine Pregnancy Test			✓			✓			✓	✓
EX	Study Drug Administration										
SDC	Study Drug Compliance			✓			✓		✓		
DAD	Dose Adjustment										
PE2	Physical Examination2			✓			✓			✓	✓
LB3	Clinical Laboratory Tests 3			✓			✓				
QS2	Questionnaires 2										✓
QS3P	Pediatric QOL Inventory PARENT RPT-INFANT 1-12M								✓	✓	✓
QS3Q	Pediatric QOL Inventory PARENT RPT-INFANT 13-24M								✓	✓	✓
QS3A	Pediatric QOL Inventory PARENT RPT (TODDLERS)								✓	✓	✓

Name	Assessment	Week 112	Week 116	Week 120	Week 124	Week 128	Week 132	Week 136	Week 140	Week 144_ET	Week 148
QS3B	Pediatric QOL Inventory PARENT RPT (YOUNG CHILD)									👍	👍
QS3C	Pediatric QOL Inventory PARENT RPT (CHILDREN)									👍	👍
QS3D	Pediatric QOL Inventory PARENT RPT (TEENS)									👍	👍
QS3E	Pediatric QOL Inventory YOUNG CHILD REPORT									👍	👍
QS3F	Pediatric QOL Inventory CHILD REPORT									👍	👍
QS3G	Pediatric QOL Inventory TEEN REPORT									👍	👍
QS3H	Multidimensional Fatigue Scale PARENT for TODDLERS									👍	👍
QS3I	Multidimensional Fatigue Scale PARENT (YOUNG CHILD)									👍	👍
QS3J	Multidimensional Fatigue Scale PARENT for CHILDREN									👍	👍
QS3K	Multidimensional Fatigue Scale PARENT for TEENS									👍	👍

Name	Assessment	Week 112	Week 116	Week 120	Week 124	Week 128	Week 132	Week 136	Week 140	Week 144_ET	Week 148
QS3L	Multidimensional Fatigue Scale YOUNG CHILD										
QS3M	Multidimensional Fatigue Scale CHILD										
QS3N	Multidimensional Fatigue Scale TEEN										
QS3O	Family Impact Module PARENT REPORT										
Q57	Xanthoma Scale										
Q55	Caregiver Impression of Change										
DS	Subject Summary										
PRE	Protocol Extension										
IE2	Inclusion/Exclusion Criteria 2										
MH2	Medical History 2										
LB	Clinical Laboratory Tests										
PRE2	Long Term Optional Treatment										
IE3	Inclusion/Exclusion Criteria 3										
DS2	Subject Summary 2										
PRE3	Long Term Optional Treatment 2										
CM	Concomitant Medications										
EX2	Study Drug Administration 2										

Name	Assessment	Week 112	Week 116	Week 120	Week 124	Week 128	Week 132	Week 136	Week 140	Week 144_ET	Week 148
AE	Adverse Events										
SDA1	Study Drug Accountability 1										
Scr	Screened										
Enroll	Enrolled										
Fail	Failed										
Disc	Discontinued										
Comp	Completed										

Name	Assessment	Long Term Optional Treatment Completion	Long Term Optional Treatment 2	Week 152	Week 156	Week 160	Week 164	Week 168	Week 172	Week 176	Week 180
DM	Informed Consent/Demography										
IE1	Inclusion/Exclusion Criteria										
MH	Medical History										
TC	Telephone Contact										
VS	Vital Signs										
LB2	Clinical Laboratory Tests 2										
QS1	Scratch Scale										
PREG2	Urine Pregnancy Test										
EX	Study Drug Administration										
SDC	Study Drug Compliance										
DAD	Dose Adjustment										
PE2	Physical Examination2										
LB3	Clinical Laboratory Tests 3										
QS2	Questionnaires 2										
QS3P	Pediatric QOL Inventory PARENT RPT - INFANT 1-12M										
QS3Q	Pediatric QOL Inventory PARENT RPT - INFANT 13-24M										
QS3A	Pediatric QOL Inventory PARENT RPT (TODDLERS)										

Name	Assessment	Long Term Optional Treatment Completion	Long Term Optional Treatment 2	Week 152	Week 156	Week 160	Week 164	Week 168	Week 172	Week 176	Week 180
QS3B	Pediatric QOL Inventory PARENT RPT (YOUNG CHILD)										
QS3C	Pediatric QOL Inventory PARENT RPT (CHILDREN)										
QS3D	Pediatric QOL Inventory PARENT RPT (TEENS)										
QS3E	Pediatric QOL Inventory YOUNG CHILD REPORT										
QS3F	Pediatric QOL Inventory CHILD REPORT										
QS3G	Pediatric QOL Inventory TEEN REPORT										
QS3H	Multidimensional Fatigue Scale PARENT for TODDLERS										
QS3I	Multidimensional Fatigue Scale PARENT (YOUNG CHILD)										
QS3J	Multidimensional Fatigue Scale PARENT for CHILDREN										
QS3K	Multidimensional Fatigue Scale PARENT for TEENS										

Name	Assessment	Long Term Optional Treatment Completion	Long Term Optional Treatment 2	Week 152	Week 156	Week 160	Week 164	Week 168	Week 172	Week 176	Week 180
QS3L	Multidimensional Fatigue Scale YOUNG CHILD										
QS3M	Multidimensional Fatigue Scale CHILD										
QS3N	Multidimensional Fatigue Scale TEEN										
QS3O	Family Impact Module PARENT REPORT										
QS7	Xanthoma Scale										
QS5	Caregiver Impression of Change										
DS	Subject Summary										
PRE	Protocol Extension										
IE2	Inclusion/Exclusion Criteria 2										
MH2	Medical History 2										
LB	Clinical Laboratory Tests										
PRE2	Long Term Optional Treatment										
IE3	Inclusion/Exclusion Criteria 3										
DS2	Subject Summary 2										
PRE3	Long Term Optional Treatment 2										
CM	Concomitant Medications										
EX2	Study Drug Administration 2										

Name	Assessment	Long Term Optional Treatment Completion	Long Term Optional Treatment 2	Week 152	Week 156	Week 160	Week 164	Week 168	Week 172	Week 176	Week 180
AE	Adverse Events										
SDA1	Study Drug Accountability 1										
Scr	Screened										
Enroll	Enrolled										
Fail	Failed										
Disc	Discontinued										
Comp	Completed										












Name	Assessment	Week 184	Week 188	Week 192	Week 196	Week 200	Week 204	Week 208	Week 212	Week 216	Week 220
DM	Informed Consent/Demography										
IE1	Inclusion/Exclusion Criteria										
MH	Medical History										
TC	Telephone Contact										
VS	Vital Signs										
LB2	Clinical Laboratory Tests 2										
QS1	Scratch Scale										
PREG2	Urine Pregnancy Test										
EX	Study Drug Administration										
SDC	Study Drug Compliance										
DAD	Dose Adjustment										
PE2	Physical Examination2										
LB3	Clinical Laboratory Tests 3										
QS2	Questionnaires 2										
QS3P	Pediatric QOL Inventory PARENT RPT-INFANT 1-12M										
QS3Q	Pediatric QOL Inventory PARENT RPT-INFANT 13-24M										
QS3A	Pediatric QOL Inventory PARENT RPT (TODDLERS)										

Name	Assessment	Week 184	Week 188	Week 192	Week 196	Week 200	Week 204	Week 208	Week 212	Week 216	Week 220
QS3B	Pediatric QOL Inventory PARENT RPT (YOUNG CHILD)									✔	✔
QS3C	Pediatric QOL Inventory PARENT RPT (CHILDREN)									✔	✔
QS3D	Pediatric QOL Inventory PARENT RPT (TEENS)									✔	✔
QS3E	Pediatric QOL Inventory YOUNG CHILD REPORT									✔	✔
QS3F	Pediatric QOL Inventory CHILD REPORT									✔	✔
QS3G	Pediatric QOL Inventory TEEN REPORT									✔	✔
QS3H	Multidimensional Fatigue Scale PARENT for TODDLERS									✔	✔
QS3I	Multidimensional Fatigue Scale PARENT (YOUNG CHILD)									✔	✔
QS3J	Multidimensional Fatigue Scale PARENT for CHILDREN									✔	✔
QS3K	Multidimensional Fatigue Scale PARENT for TEENS									✔	✔

Name	Assessment	Week 184	Week 188	Week 192	Week 196	Week 200	Week 204	Week 208	Week 212	Week 216	Week 220
QS3L	Multidimensional Fatigue Scale YOUNG CHILD										
QS3M	Multidimensional Fatigue Scale CHILD										
QS3N	Multidimensional Fatigue Scale TEEN										
QS3O	Family Impact Module PARENT REPORT										
QS7	Xanthoma Scale										
QS5	Caregiver Impression of Change										
DS	Subject Summary										
PRE	Protocol Extension										
IE2	Inclusion/Exclusion Criteria 2										
MH2	Medical History 2										
LB	Clinical Laboratory Tests										
PRE2	Long Term Optional Treatment										
IE3	Inclusion/Exclusion Criteria 3										
DS2	Subject Summary 2										
PRE3	Long Term Optional Treatment 2										
CM	Concomitant Medications										
EX2	Study Drug Administration 2										

Name	Assessment	Week 184	Week 188	Week 192	Week 196	Week 200	Week 204	Week 208	Week 212	Week 216	Week 220
AE	Adverse Events										
SDA1	Study Drug Accountability 1										
Scr	Screened										
Enroll	Enrolled										
Fail	Failed										
Disc	Discontinued										
Comp	Completed										

Name	Assessment	Long Term Optional Treatment Completion 2	Common	Unscheduled
DM	Informed Consent/Demography			
IE1	Inclusion/Exclusion Criteria			
MH	Medical History			
TC	Telephone Contact			✔
VS	Vital Signs			✔
LB2	Clinical Laboratory Tests 2			✔
QS1	Scratch Scale			✔
PREG2	Urine Pregnancy Test			✔
EX	Study Drug Administration			✔
SDC	Study Drug Compliance			✔
DAD	Dose Adjustment			
PE2	Physical Examination2			✔
LB3	Clinical Laboratory Tests 3			
QS2	Questionnaires 2			
QS3P	Pediatric QOL Inventory PARENT RPT-INFANT 1-12M			
QS3Q	Pediatric QOL Inventory PARENT RPT-INFANT 13-24M			
QS3A	Pediatric QOL Inventory PARENT RPT (TODDLERS)			
QS3B	Pediatric QOL Inventory PARENT RPT (YOUNG CHILD)			
QS3C	Pediatric QOL Inventory PARENT RPT (CHILDREN)			
QS3D	Pediatric QOL Inventory PARENT RPT (TEENS)			
QS3E	Pediatric QOL Inventory YOUNG CHILD REPORT			
QS3F	Pediatric QOL Inventory CHILD REPORT			
QS3G	Pediatric QOL Inventory TEEN REPORT			
QS3H	Multidimensional Fatigue Scale PARENT for TODDLERS			
QS3I	Multidimensional Fatigue Scale PARENT (YOUNG CHILD)			
QS3J	Multidimensional Fatigue Scale PARENT for CHILDREN			
QS3K	Multidimensional Fatigue Scale PARENT for TEENS			
QS3L	Multidimensional Fatigue Scale YOUNG CHILD			
QS3M	Multidimensional Fatigue Scale CHILD			
QS3N	Multidimensional Fatigue Scale TEEN			



Name	Assessment	Long Term Optional Treatment Completion 2	Common	Unscheduled
QS30	Family Impact Module PARENT REPORT			
QS7	Xanthoma Scale			
QS5	Caregiver Impression of Change			
DS	Subject Summary			
PRE	Protocol Extension			
IE2	Inclusion/Exclusion Criteria 2			
MH2	Medical History 2			
LB	Clinical Laboratory Tests			
PRE2	Long Term Optional Treatment			
IE3	Inclusion/Exclusion Criteria 3			
DS2	Subject Summary 2			
PRE3	Long Term Optional Treatment 2			
CM	Concomitant Medications			
EX2	Study Drug Administration 2			
AE	Adverse Events			
SDA1	Study Drug Accountability 1			
Scr	Screened			
Enroll	Enrolled			
Fail	Failed			
Disc	Discontinued			
Comp	Completed			

Event Labels	Names
Day 0	Day0
Week 1	Week1
Week 2	Week2
Week 3	Week3
Week 4	Week4
Week 6	Week6
Week 8	Week8
Week 10	Week10
Week 12	Week12
Week 16	Week16
Week 20	Week20
Week 24	Week24
Week 28	Week28
Week 32	Week32
Week 36	Week36
Week 40	Week40
Week 44	Week44
Week 48	Week48_ET
Post-Week 48 Follow-up	FollowUp
Study Completion (Complete)	StudyCompletion
Study Completion (Discontinue)	Study_Completion
Re-Initiation Day 0	RID0
Re-Initiation Week 49	RIW49
Re-Initiation Week 50	RIW50
Re-Initiation Week 51	RIW51
Re-Initiation Week 52	RIW52
Week 52	Week52
Week 56	Week56
Week 60	Week60
Week 64	Week64
Week 68	Week68
Week 72	Week72
Week 76	Week76
Week 80	Week80
Week 84	Week84
Week 88	Week88
Week 92	Week92
Week 96_ET	Week96_ET
30 Day Follow-up	30D_FU
Extension Completion	EC
Long Term Optional Treatment	PA5
PA5 Re-Initiation Day -2	PA5_RID2
PA5 Re-Initiation Day 0	PA5_RID0
PA5 Re-Initiation Week 1	PA5_RIW1
PA5 Re-Initiation Week 2	PA5_RIW2
PA5 Re-Initiation Week 3	PA5_RIW3
PA5 Re-Initiation Week 4	PA5_RIW4
Week 100	W100


Event Labels	Names
Week 104	W104
Week 108	W108
Week 112	W112
Week 116	W116
Week 120	W120
Week 124	W124
Week 128	W128
Week 132	W132
Week 136	W136
Week 140	W140
Week 144_ET	W144_ET
Week 148	W148
Long Term Optional Treatment Completion	PA5_EC
Long Term Optional Treatment 2	PA6
Week 152	W152
Week 156	W156
Week 160	W160
Week 164	W164
Week 168	W168
Week 172	W172
Week 176	W176
Week 180	W180
Week 184	W184
Week 188	W188
Week 192	W192
Week 196	W196
Week 200	W200
Week 204	W204
Week 208	W208
Week 212	W212
Week 216	W216
Week 220	W220
Long Term Optional Treatment Completion 2	PA6_EC
Common	Common
Unscheduled	Unscheduled

Informed Consent/Demography (DM)

INFORMED CONSENT

1.	Date Parental Informed Consent signed	<input type="text"/>	 dd-mmm-yyyy
2.	Young Adult Consent Not Applicable	List: NA <input type="text" value="6"/>	
**3.	Date Young Adult Consent signed	<input type="text"/>	 dd-mmm-yyyy
4.	Assent Not Applicable	List: NA <input type="text" value="6"/>	
**5.	Date Assent signed	<input type="text"/>	 dd-mmm-yyyy

DEMOGRAPHY

6.	Birth date	<input type="text"/>	 dd-mmm-yyyy
	*6.1. Age	Read Only: Derived	
	*6.2. Age Units	Read Only: Derived	
7.	Sex	List: SEX <input type="text" value="6"/>	
	*7.1. Country	Read Only: Derived	
8.	Weight (kg)	<input type="text"/>	
	*8.1. Weight Units	List: WEIGHT <input type="text" value="6"/>	
	* Hidden Question		
	** Conditional Question		

List:NA	
Label	Value
[Blank]	
Not Applicable	NA

List:SEX	
Label	Value
[Blank]	
Male	M
Female	F

List:WEIGHT	
Label	Value
kg	kg

Inclusion/Exclusion Criteria (IE1)

1.	Protocol Criteria Version	List: TIVERS 6																														
2.	Did the subject meet all eligibility criteria?	List: YES_NO 6																														
3.	Inclusion/Exclusion Criteria																															
3.R.	<table border="1"> <thead> <tr> <th>Criterion Type</th> <th>Criterion not met</th> <th>Specify</th> <th>Approval date</th> <th>Sponsor approval?</th> </tr> </thead> <tbody> <tr> <td>3.R.1. Criterion Type</td> <td></td> <td></td> <td>List: IE 6</td> <td></td> </tr> <tr> <td>3.R.2. Criterion not met</td> <td></td> <td></td> <td>List: IENUM 6</td> <td></td> </tr> <tr> <td>3.R.3. Specify</td> <td></td> <td></td> <td></td> <td></td> </tr> <tr> <td>**3.R.4. Date of approval</td> <td></td> <td></td> <td></td> <td>dd-mmm-yyyy</td> </tr> <tr> <td>3.R.5. Lumena approval received?</td> <td></td> <td></td> <td>List: YES_NO 6</td> <td></td> </tr> </tbody> </table>	Criterion Type	Criterion not met	Specify	Approval date	Sponsor approval?	3.R.1. Criterion Type			List: IE 6		3.R.2. Criterion not met			List: IENUM 6		3.R.3. Specify					**3.R.4. Date of approval				dd-mmm-yyyy	3.R.5. Lumena approval received?			List: YES_NO 6		
Criterion Type	Criterion not met	Specify	Approval date	Sponsor approval?																												
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3.R.2. Criterion not met			List: IENUM 6																													
3.R.3. Specify																																
**3.R.4. Date of approval				dd-mmm-yyyy																												
3.R.5. Lumena approval received?			List: YES_NO 6																													
Add New Row																																

** Conditional Question

List:TIVERS		List:YES_NO		List:IE	
Label	Value	Label	Value	Label	Value
[Blank]		[Blank]		[Blank]	
Original - 14APR2013	Original - 14APR2013	Yes	Y	Inclusion	1
Amendment 1 - 29JAN2015	Amendment 1 - 29JAN2015	No	N	Exclusion	2
Amendment 2 - 12FEB2015	Amendment 2 - 12FEB2015				
Amendment 4 - 27APR2016	Amendment 4 - 27APR2016				
Amendment 5 - 13NOV2017	Amendment 5 - 13NOV2017				

List:IENUM	
Label	Value
[Blank]	
1	1
2	2
3	3
4	4
5	5
6	6
7	7
8	8
9	9
10	10

Medical History (MH)

1. Any new significant medical history?

List: YES_NO 6

2. Medical History

2.R.	Verbatim Term	Start Date (Day)	Start Date (Month)	Start Date (Year)	Ongoing
2.R.1.	Verbatim term				
*2.R.1.1.	MHTERM_Enc_TERM				Read Only: Encoded
*2.R.1.2.	MHTERM_Enc_CODE				Read Only: Encoded
*2.R.1.3.	MHTERM_Enc_TYPE				Read Only: Encoded
*2.R.1.4.	MHTERM_Enc_CAT1				Read Only: Encoded
*2.R.1.5.	MHTERM_Enc_CAT2				Read Only: Encoded
*2.R.1.6.	MHTERM_Enc_CAT3				Read Only: Encoded
*2.R.1.7.	MHTERM_Enc_CAT4				Read Only: Encoded
*2.R.1.8.	MHTERM_Enc_CAT5				Read Only: Encoded
*2.R.1.9.	MHTERM_Enc_CAT6				Read Only: Encoded
*2.R.1.10.	MHTERM_Enc_CAT7				Read Only: Encoded
*2.R.1.11.	MHTERM_Enc_CAT8				Read Only: Encoded
*2.R.1.12.	MHTERM_Enc_CAT9				Read Only: Encoded
2.R.2.	Start Date (Day)				List: DAY 6
2.R.3.	Start Date (Month)				List: MONTH 6
2.R.4.	Start Date (Year)				
*2.R.4.1.	Start Date				Read Only: Derived
2.R.5.	Ongoing?				List: YES 6
**2.R.6.	End Date (Day)				List: DAY 6
**2.R.7.	End Date (Month)				List: MONTH 6
**2.R.8.	End Date (Year)				
*2.R.8.1.	End Date				Read Only: Derived

Add New Row

* Hidden Question

** Conditional Question

List: YES_NO		List: DAY		List: MONTH	
Label	Value	Label	Value	Label	Value
[Blank]		[Blank]		[Blank]	
Yes	Y	01	01	JAN	JAN
No	N	02	02	FEB	FEB
		03	03	MAR	MAR
		04	04	APR	APR
		05	05	MAY	MAY
		06	06	JUN	JUN
		07	07	JUL	JUL

08	08	AUG	AUG
09	09	SEP	SEP
10	10	OCT	OCT
11	11	NOV	NOV
12	12	DEC	DEC
13	13	UNK	UNK
14	14		
15	15		
16	16		
17	17		
18	18		
19	19		
20	20		
21	21		
22	22		
23	23		
24	24		
25	25		
26	26		
27	27		
28	28		
29	29		
30	30		
31	31		
UN	UN		

List: YES	
Label	Value
[Blank]	
YES	Y

Telephone Contact (TC)

1. Was subject/caregiver contacted by telephone?

List: YES_NO 6

**2. Date of telephone contact

dd-mm-yyyy

** Conditional Question

List: YES_NO	
Label	Value
[Blank]	
Yes	Y
No	N

Vital Signs (VS)

1. Were vital signs collected?	List: YES_NO 6
**2. Date of measurements	<input type="text"/> dd-mm-yyyy
**3. Height (cm)	<input type="text"/>
*3.1. Height Unit	List: HEIGHT 6
**4. Weight (kg)	<input type="text"/>
*4.1. Weight Unit	List: WEIGHT 6
**5. Temperature (C)	<input type="text"/>
*5.1. Temperature Unit	List: TEMP 6
**6. Heart Rate (bpm)	<input type="text"/>
*6.1. Heart Rate Unit	List: bpm 6
**7. Respiratory Rate (rpm)	<input type="text"/>
*7.1. Respiratory Rate Unit	List: rpm 6
**8. Systolic Blood Pressure (mmHg)	<input type="text"/>
**9. Diastolic Blood Pressure (mmHg)	<input type="text"/>
*9.1. Blood Pressure Unit	List: mmHg 6

* Hidden Question

** Conditional Question





List: YES_NO		List: HEIGHT		List: WEIGHT	
Label	Value	Label	Value	Label	Value
[Blank]		cm	cm	kg	kg
Yes	Y				
No	N				

List: TEMP		List: bpm		List: rpm	
Label	Value	Label	Value	Label	Value
C	C	BEATS/MIN	BEATS/MIN	rpm	rpm

List: mmHg	
Label	Value
mmHg	mmHg

Clinical Laboratory Tests 2 (LB2)

1. Clinical Laboratory Tests

1.R.	Sample Type	Sample collected?	Collection Date	Sample ID
1.1.1.	Sample Type			Blood
1.1.2.	Was the sample collected?			List: YES_NO 6
**1.1.3.	Collection Date		<input type="text"/>	 dd-mmm-yyyy
**1.1.4.	Sample ID		<input type="text"/>	
1.2.1.	Sample Type			Urine
1.2.2.	Was the sample collected?			List: YES_NO 6
**1.2.3.	Collection Date		<input type="text"/>	 dd-mmm-yyyy
**1.2.4.	Sample ID		<input type="text"/>	
2.	Date of last dose prior to sample collection		<input type="text"/>	 dd-mmm-yyyy
3.	Time of last dose prior to sample collection (hh:mm, 24-hour clock)		<input type="text"/>	
4.	Date of last meal prior to sample collection		<input type="text"/>	 dd-mmm-yyyy
5.	Time of last meal prior to sample collection (hh:mm, 24-hour clock)		<input type="text"/>	

** Conditional Question

List: YES_NO	
Label	Value
[Blank]	
Yes	Y
No	N

Scratch Scale (QS1)

1. Was Clinician Scratch Scale completed?

List: YES_NO 6

**2. Date of assessment

 dd-mm-yyyy

**3. Clinician Scratch Scale score

List: SCRATCH 6

** Conditional Question

List: YES_NO		List: SCRATCH	
Label	Value	Label	Value
[Blank]		[Blank]	
Yes	Y	0 - None	0
No	N	1 - Rubbing or mild scratching when undistracted	1
		2 - Active scratching without evident skin abrasions	2
		3 - Abrasion evident	3
		4 - Cutaneous mutilation, haemorrhage and scarring evident	4

Urine Pregnancy Test (PREG2)

1.	Sample Type	List: UPREG 6
2.	Was the sample collected?	List: Y_N_NA 6
**3.	Date collected	<input type="text"/> dd-mm-yyyy
**4.	Results	List: POSNEG 6

** Conditional Question

List:UPREG	
Label	Value
Urine	Urine

List:Y_N_NA	
Label	Value
[Blank]	
Yes	Y
No	N
Not Applicable	NA

List:POSNEG	
Label	Value
[Blank]	
Positive	+
Negative	-

Study Drug Administration (EX)

1. Date of First Dose

  dd-mmm-yyyy

2. Volume of study drug solution (mL)

List: EXVOL_VAL **List:EXVOL_VAL**

Label	Value
[Blank]	
0.5	0.5
1.0	1.0

Study Drug Compliance (SDC)

1. Was study drug taken as prescribed?

List: YES_NO 6

2. Date(s) of Dosing Error

2.R. Start Date End Date Comments Type of dosing error

2.R.1. Start Date of dosing error

 dd-mm-yyyy

2.R.2. End Date of dosing error

 dd-mm-yyyy

2.R.3. Comments

2.R.4. Type of dosing error

List: EXMD 6

[Add New Row](#)**List: YES_NO**

Label	Value
[Blank]	
Yes	Y
No	N

List: EXMD

Label	Value
[Blank]	
Missed Dose	1
Underdose	2
Overdose	3
Other	4
Protocol Amendment	5
Gap	

Dose Adjustment (DAD)

1. Was the dose adjusted at Week 12?

List: YES_NO 

List: YES_NO	
Label	Value
[Blank]	
Yes	Y
No	N

Physical Examination2 (PE2)

1. Was the physical examination performed?

List: YES_NO 6

**2. Exam Date

dd-mm-yyyy

**3. Are there any clinically significant abnormal findings that have been newly diagnosed or have worsened since the previous assessment ?

List: YES_NO 6

** Conditional Question

List: YES_NO	
Label	Value
[Blank]	
Yes	Y
No	N

Clinical Laboratory Tests 3 (LB3)

1. Clinical Laboratory Tests

1.R.	Sample Type	Sample collected?	Collection Date	Sample ID
1.1.1.	Sample Type			Blood
1.1.2.	Was the sample collected?			List: YES_NO 6
**1.1.3.	Collection Date			<input type="text"/> dd-mmm-yyyy
**1.1.4.	Sample ID			<input type="text"/>
1.2.1.	Sample Type			Urine
1.2.2.	Was the sample collected?			List: YES_NO 6
**1.2.3.	Collection Date			<input type="text"/> dd-mmm-yyyy
**1.2.4.	Sample ID			<input type="text"/>
2.	Date of last meal prior to sample collection			<input type="text"/> dd-mmm-yyyy
3.	Time of last meal prior to sample collection (hh:mm, 24-hour clock)			<input type="text"/>

** Conditional Question

List: YES_NO	
Label	Value
[Blank]	
Yes	Y
No	N

Questionnaires 2 (QS2)

1.	Was the Pediatric QoL Inventory PARENT REPORT completed by caregiver?	List: YES_NO 6
**2.	Date Pediatric QoL Inventory PARENT REPORT completed by caregiver	<input type="text"/> dd-mm-yyyy
**3.	For which age category was the questionnaire completed?	List: AGE1 6
4.	Was the Pediatric QoL Inventory completed by subject?	List: Y_N_NA 6
**5.	Date Pediatric QoL Inventory completed by subject	<input type="text"/> dd-mm-yyyy
**6.	For which age category was the questionnaire completed?	List: AGE2 6
7.	Was the Multidimensional Fatigue Scale PARENT REPORT completed by caregiver?	List: Y_N_NA 6
**8.	Date Multidimensional Fatigue Scale PARENT REPORT completed by caregiver	<input type="text"/> dd-mm-yyyy
**9.	For which age category was the questionnaire completed?	List: AGE3 6
10.	Was the Multidimensional Fatigue Scale completed by subject?	List: Y_N_NA 6
**11.	Date Multidimensional Fatigue Scale completed by subject	<input type="text"/> dd-mm-yyyy
**12.	For which age category was the questionnaire completed?	List: AGE2 6
13.	Was the Family Impact Module PARENT REPORT completed by caregiver?	List: YES_NO 6
**14.	Date Family Impact Module PARENT REPORT completed by caregiver	<input type="text"/> dd-mm-yyyy

** Conditional Question

List: YES_NO		List: AGE1		List: Y_N_NA	
Label	Value	Label	Value	Label	Value
[Blank]		[Blank]		[Blank]	
Yes	Y	INFANTS (1-12 months)	5	Yes	Y
No	N	INFANTS (13-24 months)	6	No	N
		TODDLERS (ages 2-4)	1	Not Applicable	NA
		YOUNG CHILDREN (ages 5-7)	2		
		CHILDREN (ages 8-12)	3		
		TEENAGERS (ages 13-18)	4		

List: AGE2		List: AGE3	
Label	Value	Label	Value
[Blank]		[Blank]	
YOUNG CHILD REPORT (ages 5-7)	2	TODDLERS (ages 2-4)	1
		YOUNG CHILDREN (ages 5-7)	2

REPORT (ages 8-12)	3	CHILDREN (ages 8-12)	3
TEENAGER REPORT (ages 13-18)	4	TEENAGERS (ages 13-18)	4

Pediatric QOL Inventory PARENT RPT-INFANT 1-12M (QS3P)

In the past **ONE month**, how much of a **problem** has your child had with...

PHYSICAL FUNCTIONING (problems with...)

1.	1. Low energy level	List: PEDSQL1 <input type="text" value="6"/>
2.	2. Difficulty participating in active play	List: PEDSQL1 <input type="text" value="6"/>
3.	3. Having hurts or aches	List: PEDSQL1 <input type="text" value="6"/>
4.	4. Feeling tired	List: PEDSQL1 <input type="text" value="6"/>
5.	5. Being lethargic	List: PEDSQL1 <input type="text" value="6"/>
6.	6. Resting a lot	List: PEDSQL1 <input type="text" value="6"/>

PHYSICAL SYMPTOMS (problems with...)

7.	1. Having gas	List: PEDSQL1 <input type="text" value="6"/>
8.	2. Spitting up after eating	List: PEDSQL1 <input type="text" value="6"/>
9.	3. Difficulty breathing	List: PEDSQL1 <input type="text" value="6"/>
10.	4. Being sick to his/her stomach	List: PEDSQL1 <input type="text" value="6"/>
11.	5. Difficulty Swallowing	List: PEDSQL1 <input type="text" value="6"/>
12.	6. Being constipated	List: PEDSQL1 <input type="text" value="6"/>
13.	7. Having a rash	List: PEDSQL1 <input type="text" value="6"/>
14.	8. Having diarrhea	List: PEDSQL1 <input type="text" value="6"/>
15.	9. Wheezing	List: PEDSQL1 <input type="text" value="6"/>
16.	10. Vomiting	List: PEDSQL1 <input type="text" value="6"/>

EMOTIONAL FUNCTIONING (problems with...)

17.	1. Feeling afraid or scared	List: PEDSQL1 <input type="text" value="6"/>
18.	2. Feeling angry	List: PEDSQL1 <input type="text" value="6"/>
19.	3. Crying or fussing when left alone	List: PEDSQL1 <input type="text" value="6"/>
20.	4. Difficulty soothing himself/herself when upset	List: PEDSQL1 <input type="text" value="6"/>
21.	5. Difficulty falling asleep	List: PEDSQL1 <input type="text" value="6"/>
22.	6. Crying or fussing while being cuddled	List: PEDSQL1 <input type="text" value="6"/>
23.	7. Feeling sad	List: PEDSQL1 <input type="text" value="6"/>
24.	8. Difficulty being soothed when picked up or held	List: PEDSQL1 <input type="text" value="6"/>
25.	9. Difficulty sleeping mostly through the night	List: PEDSQL1 <input type="text" value="6"/>
26.	10. Crying a lot	List: PEDSQL1 <input type="text" value="6"/>
27.	11. Feeling cranky	List: PEDSQL1 <input type="text" value="6"/>
28.	12. Difficulty taking naps during the day	List: PEDSQL1 <input type="text" value="6"/>

SOCIAL FUNCTIONING (problems with...)

29.	1. Not smiling at others	List: PEDSQL1 <input type="text" value="6"/>
30.	2. Not laughing when tickled	List: PEDSQL1 <input type="text" value="6"/>

31.	3. Not making eye contact with a caregiver	List: PEDSQL1 <input type="text" value="6"/>
32.	4. Not laughing when cuddled	List: PEDSQL1 <input type="text" value="6"/>
COGNITIVE FUNCTIONING (<i>problems with...</i>)		
33.	1. Not imitating caregivers\' actions	List: PEDSQL1 <input type="text" value="6"/>
34.	2. Not imitating caregivers\' facial expressions	List: PEDSQL1 <input type="text" value="6"/>
35.	3. Not imitating caregivers\' sounds	List: PEDSQL1 <input type="text" value="6"/>
36.	4. Not able to fix his/her attention on objects	List: PEDSQL1 <input type="text" value="6"/>

List:PEDSQL1

Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Pediatric QOL Inventory PARENT RPT-INFANT 13-24M (QS3Q)

In the past **ONE month**, how much of a **problem** has your child had with...

PHYSICAL FUNCTIONING (problems with...)

1.	1. Low energy level	List: PEDSQL1 <input type="text" value="6"/>
2.	2. Difficulty participating in active play	List: PEDSQL1 <input type="text" value="6"/>
3.	3. Having hurts or aches	List: PEDSQL1 <input type="text" value="6"/>
4.	4. Feeling tired	List: PEDSQL1 <input type="text" value="6"/>
5.	5. Being lethargic	List: PEDSQL1 <input type="text" value="6"/>
6.	6. Resting a lot	List: PEDSQL1 <input type="text" value="6"/>
7.	7. Feeling too tired to play	List: PEDSQL1 <input type="text" value="6"/>
8.	8. Difficulty walking	List: PEDSQL1 <input type="text" value="6"/>
9.	9. Difficulty running a short distance without falling	List: PEDSQL1 <input type="text" value="6"/>

PHYSICAL SYMPTOMS (problems with...)

10.	1. Having gas	List: PEDSQL1 <input type="text" value="6"/>
11.	2. Spitting up after eating	List: PEDSQL1 <input type="text" value="6"/>
12.	3. Difficulty breathing	List: PEDSQL1 <input type="text" value="6"/>
13.	4. Being sick to his/her stomach	List: PEDSQL1 <input type="text" value="6"/>
14.	5. Difficulty swallowing	List: PEDSQL1 <input type="text" value="6"/>
15.	6. Being constipated	List: PEDSQL1 <input type="text" value="6"/>
16.	7. Having a rash	List: PEDSQL1 <input type="text" value="6"/>
17.	8. Having diarrhea	List: PEDSQL1 <input type="text" value="6"/>
18.	9. Wheezing	List: PEDSQL1 <input type="text" value="6"/>
19.	10. Vomiting	List: PEDSQL1 <input type="text" value="6"/>

EMOTIONAL FUNCTIONING (problems with...)

20.	1. Feeling afraid or scared	List: PEDSQL1 <input type="text" value="6"/>
21.	2. Feeling angry	List: PEDSQL1 <input type="text" value="6"/>
22.	3. Crying or fussing when left alone	List: PEDSQL1 <input type="text" value="6"/>
23.	4. Difficulty soothing himself/herself when upset	List: PEDSQL1 <input type="text" value="6"/>
24.	5. Difficulty falling asleep	List: PEDSQL1 <input type="text" value="6"/>
25.	6. Crying or fussing while being cuddled	List: PEDSQL1 <input type="text" value="6"/>
26.	7. Feeling sad	List: PEDSQL1 <input type="text" value="6"/>
27.	8. Difficulty being soothed when picked up or held	List: PEDSQL1 <input type="text" value="6"/>
28.	9. Difficulty sleeping mostly through the night	List: PEDSQL1 <input type="text" value="6"/>
29.	10. Crying a lot	List: PEDSQL1 <input type="text" value="6"/>
30.	11. Feeling cranky	List: PEDSQL1 <input type="text" value="6"/>
31.	12. Difficulty taking naps during the day	List: PEDSQL1 <input type="text" value="6"/>

SOCIAL FUNCTIONING (*problems with...*)

32.	1. Not smiling at others	List: PEDSQL1 <input type="text" value="6"/>
33.	2. Not laughing when tickled	List: PEDSQL1 <input type="text" value="6"/>
34.	3. Not making eye contact with a caregiver	List: PEDSQL1 <input type="text" value="6"/>
35.	4. Not laughing when cuddled	List: PEDSQL1 <input type="text" value="6"/>
36.	5. Being uncomfortable around other children	List: PEDSQL1 <input type="text" value="6"/>

COGNITIVE FUNCTIONING (*problems with...*)

37.	1. Not imitating caregivers' actions	List: PEDSQL1 <input type="text" value="6"/>
38.	2. Not imitating caregivers' facial expressions	List: PEDSQL1 <input type="text" value="6"/>
39.	3. Not imitating caregivers' sounds	List: PEDSQL1 <input type="text" value="6"/>
40.	4. Not able to fix his/her attention on objects	List: PEDSQL1 <input type="text" value="6"/>
41.	5. Not imitating caregivers' speech	List: PEDSQL1 <input type="text" value="6"/>
42.	6. Difficulty pointing to his/her body parts when asked	List: PEDSQL1 <input type="text" value="6"/>
43.	7. Difficulty naming familiar objects	List: PEDSQL1 <input type="text" value="6"/>
44.	8. Difficulty repeating words	List: PEDSQL1 <input type="text" value="6"/>
45.	9. Difficulty keeping his/her attention on things	List: PEDSQL1 <input type="text" value="6"/>

List:PEDSQL1

Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Pediatric QOL Inventory PARENT RPT (TODDLERS) (QS3A)

In the past **ONE month**, how much of a **problem** has your child had with...

PHYSICAL FUNCTIONING (problems with...)

1.	1. Walking	List: PEDSQL1 <input type="text" value="6"/>
2.	2. Running	List: PEDSQL1 <input type="text" value="6"/>
3.	3. Participating in active play or exercise	List: PEDSQL1 <input type="text" value="6"/>
4.	4. Lifting something heavy	List: PEDSQL1 <input type="text" value="6"/>
5.	5. Bathing	List: PEDSQL1 <input type="text" value="6"/>
6.	6. Helping to pick up his or her toys	List: PEDSQL1 <input type="text" value="6"/>
7.	7. Having hurts or aches	List: PEDSQL1 <input type="text" value="6"/>
8.	8. Low energy level	List: PEDSQL1 <input type="text" value="6"/>

EMOTIONAL FUNCTIONING (problems with...)

9.	1. Feeling afraid or scared	List: PEDSQL1 <input type="text" value="6"/>
10.	2. Feeling sad or blue	List: PEDSQL1 <input type="text" value="6"/>
11.	3. Feeling angry	List: PEDSQL1 <input type="text" value="6"/>
12.	4. Trouble sleeping	List: PEDSQL1 <input type="text" value="6"/>
13.	5. Worrying	List: PEDSQL1 <input type="text" value="6"/>

SOCIAL FUNCTIONING (problems with...)

14.	1. Playing with other children	List: PEDSQL1 <input type="text" value="6"/>
15.	2. Other kids not wanting to play with him or her	List: PEDSQL1 <input type="text" value="6"/>
16.	3. Getting teased by other children	List: PEDSQL1 <input type="text" value="6"/>
17.	4. Not able to do things that other children his or her age can do	List: PEDSQL1 <input type="text" value="6"/>
18.	5. Keeping up when playing with other children	List: PEDSQL1 <input type="text" value="6"/>

***Please complete this section if your child attends school or daycare**

SCHOOL FUNCTIONING (problems with...)

19.	1. Doing the same school activities as peers	List: PEDSQL1 <input type="text" value="6"/>
20.	2. Missing school/daycare because of not feeling well	List: PEDSQL1 <input type="text" value="6"/>
21.	3. Missing school/daycare to go to the doctor or hospital	List: PEDSQL1 <input type="text" value="6"/>

List: PEDSQL1	
Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Pediatric QOL Inventory PARENT RPT (YOUNG CHILD) (QS3B)

In the past **ONE month**, how much of a **problem** has your child had with...

PHYSICAL FUNCTIONING (problems with...)

1.	1. Walking more than one block	List: PEDSQL1 <input type="text" value="6"/>
2.	2. Running	List: PEDSQL1 <input type="text" value="6"/>
3.	3. Participating in sports activity or exercise	List: PEDSQL1 <input type="text" value="6"/>
4.	4. Lifting something heavy	List: PEDSQL1 <input type="text" value="6"/>
5.	5. Taking a bath or shower by him or herself	List: PEDSQL1 <input type="text" value="6"/>
6.	6. Doing chores, like picking up his or her toys	List: PEDSQL1 <input type="text" value="6"/>
7.	7. Having hurts or aches	List: PEDSQL1 <input type="text" value="6"/>
8.	8. Low energy level	List: PEDSQL1 <input type="text" value="6"/>

EMOTIONAL FUNCTIONING (problems with...)

9.	1. Feeling afraid or scared	List: PEDSQL1 <input type="text" value="6"/>
10.	2. Feeling sad or blue	List: PEDSQL1 <input type="text" value="6"/>
11.	3. Feeling angry	List: PEDSQL1 <input type="text" value="6"/>
12.	4. Trouble sleeping	List: PEDSQL1 <input type="text" value="6"/>
13.	5. Worrying about what will happen to him or her	List: PEDSQL1 <input type="text" value="6"/>

SOCIAL FUNCTIONING (problems with...)

14.	1. Getting along with other children	List: PEDSQL1 <input type="text" value="6"/>
15.	2. Other kids not wanting to be his or her friend	List: PEDSQL1 <input type="text" value="6"/>
16.	3. Getting teased by other children	List: PEDSQL1 <input type="text" value="6"/>
17.	4. Not able to do things that other children his or her age can do	List: PEDSQL1 <input type="text" value="6"/>
18.	5. Keeping up when playing with other children	List: PEDSQL1 <input type="text" value="6"/>

SCHOOL FUNCTIONING (problems with...)

19.	1. Paying attention in class	List: PEDSQL1 <input type="text" value="6"/>
20.	2. Forgetting things	List: PEDSQL1 <input type="text" value="6"/>
21.	3. Keeping up with school activities	List: PEDSQL1 <input type="text" value="6"/>
22.	4. Missing school because of not feeling well	List: PEDSQL1 <input type="text" value="6"/>
23.	5. Missing school to go to the doctor or hospital	List: PEDSQL1 <input type="text" value="6"/>

List: PEDSQL1

Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Pediatric QOL Inventory PARENT RPT (CHILDREN) (QS3C)

In the past **ONE month**, how much of a **problem** has your child had with...

PHYSICAL FUNCTIONING (problems with...)

1.	1. Walking more than one block	List: PEDSQL1 <input type="text" value="6"/>
2.	2. Running	List: PEDSQL1 <input type="text" value="6"/>
3.	3. Participating in sports activity or exercise	List: PEDSQL1 <input type="text" value="6"/>
4.	4. Lifting something heavy	List: PEDSQL1 <input type="text" value="6"/>
5.	5. Taking a bath or shower by him or herself	List: PEDSQL1 <input type="text" value="6"/>
6.	6. Doing chores around the house	List: PEDSQL1 <input type="text" value="6"/>
7.	7. Having hurts or aches	List: PEDSQL1 <input type="text" value="6"/>
8.	8. Low energy level	List: PEDSQL1 <input type="text" value="6"/>

EMOTIONAL FUNCTIONING (problems with...)

9.	1. Feeling afraid or scared	List: PEDSQL1 <input type="text" value="6"/>
10.	2. Feeling sad or blue	List: PEDSQL1 <input type="text" value="6"/>
11.	3. Feeling angry	List: PEDSQL1 <input type="text" value="6"/>
12.	4. Trouble sleeping	List: PEDSQL1 <input type="text" value="6"/>
13.	5. Worrying about what will happen to him or her	List: PEDSQL1 <input type="text" value="6"/>

SOCIAL FUNCTIONING (problems with...)

14.	1. Getting along with other children	List: PEDSQL1 <input type="text" value="6"/>
15.	2. Other kids not wanting to be his or her friend	List: PEDSQL1 <input type="text" value="6"/>
16.	3. Getting teased by other children	List: PEDSQL1 <input type="text" value="6"/>
17.	4. Not able to do things that other children his or her age can do	List: PEDSQL1 <input type="text" value="6"/>
18.	5. Keeping up when playing with other children	List: PEDSQL1 <input type="text" value="6"/>

SCHOOL FUNCTIONING (problems with...)

19.	1. Paying attention in class	List: PEDSQL1 <input type="text" value="6"/>
20.	2. Forgetting things	List: PEDSQL1 <input type="text" value="6"/>
21.	3. Keeping up with schoolwork	List: PEDSQL1 <input type="text" value="6"/>
22.	4. Missing school because of not feeling well	List: PEDSQL1 <input type="text" value="6"/>
23.	5. Missing school to go to the doctor or hospital	List: PEDSQL1 <input type="text" value="6"/>

List: PEDSQL1

Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Pediatric QOL Inventory PARENT RPT (TEENS) (QS3D)

In the past **ONE month**, how much of a **problem** has your teen had with...

PHYSICAL FUNCTIONING (problems with...)

1.	1. Walking more than one block	List: PEDSQL1 <input type="text" value="6"/>
2.	2. Running	List: PEDSQL1 <input type="text" value="6"/>
3.	3. Participating in sports activity or exercise	List: PEDSQL1 <input type="text" value="6"/>
4.	4. Lifting something heavy	List: PEDSQL1 <input type="text" value="6"/>
5.	5. Taking a bath or shower by him or herself	List: PEDSQL1 <input type="text" value="6"/>
6.	6. Doing chores around the house	List: PEDSQL1 <input type="text" value="6"/>
7.	7. Having hurts or aches	List: PEDSQL1 <input type="text" value="6"/>
8.	8. Low energy level	List: PEDSQL1 <input type="text" value="6"/>

EMOTIONAL FUNCTIONING (problems with...)

9.	1. Feeling afraid or scared	List: PEDSQL1 <input type="text" value="6"/>
10.	2. Feeling sad or blue	List: PEDSQL1 <input type="text" value="6"/>
11.	3. Feeling angry	List: PEDSQL1 <input type="text" value="6"/>
12.	4. Trouble sleeping	List: PEDSQL1 <input type="text" value="6"/>
13.	5. Worrying about what will happen to him or her	List: PEDSQL1 <input type="text" value="6"/>

SOCIAL FUNCTIONING (problems with...)

14.	1. Getting along with other teens	List: PEDSQL1 <input type="text" value="6"/>
15.	2. Other teens not wanting to be his or her friend	List: PEDSQL1 <input type="text" value="6"/>
16.	3. Getting teased by other teens	List: PEDSQL1 <input type="text" value="6"/>
17.	4. Not able to do things that other teens his or her age can do	List: PEDSQL1 <input type="text" value="6"/>
18.	5. Keeping up with other teens	List: PEDSQL1 <input type="text" value="6"/>

SCHOOL FUNCTIONING (problems with...)

19.	1. Paying attention in class	List: PEDSQL1 <input type="text" value="6"/>
20.	2. Forgetting things	List: PEDSQL1 <input type="text" value="6"/>
21.	3. Keeping up with schoolwork	List: PEDSQL1 <input type="text" value="6"/>
22.	4. Missing school because of not feeling well	List: PEDSQL1 <input type="text" value="6"/>
23.	5. Missing school to go to the doctor or hospital	List: PEDSQL1 <input type="text" value="6"/>

List: PEDSQL1

Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Pediatric QOL Inventory YOUNG CHILD REPORT (QS3E)

Think about how you have been doing for the last few weeks. Please listen carefully to each sentence and tell me how much of a problem this is for you.

After reading the item, gesture to the template. If the child hesitates or does not seem to understand how to answer, read the response options while pointing at the faces.

PHYSICAL FUNCTIONING (problems with...)

- | | |
|--|--|
| 1. 1. Is it hard for you to walk | List: PEDSQL2 <input type="text" value="6"/> |
| 2. 2. Is it hard for you to run | List: PEDSQL2 <input type="text" value="6"/> |
| 3. 3. Is it hard for you to play sports or exercise | List: PEDSQL2 <input type="text" value="6"/> |
| 4. 4. Is it hard for you to pick up big things | List: PEDSQL2 <input type="text" value="6"/> |
| 5. 5. Is it hard for you to take a bath or shower | List: PEDSQL2 <input type="text" value="6"/> |
| 6. 6. Is it hard for you to do chores (like pick up your toys) | List: PEDSQL2 <input type="text" value="6"/> |
| 7. 7. Do you have hurts or aches | List: PEDSQL2 <input type="text" value="6"/> |
| 8. Where? | <input type="text"/> |
| 9. 8. Do you ever feel too tired to play | List: PEDSQL2 <input type="text" value="6"/> |

Remember, tell me how much of a problem this has been for you for the last few weeks.

EMOTIONAL FUNCTIONING (problems with...)

- | | |
|---|--|
| 10. 1. Do you feel scared | List: PEDSQL2 <input type="text" value="6"/> |
| 11. 2. Do you feel sad | List: PEDSQL2 <input type="text" value="6"/> |
| 12. 3. Do you feel mad | List: PEDSQL2 <input type="text" value="6"/> |
| 13. 4. Do you have trouble sleeping | List: PEDSQL2 <input type="text" value="6"/> |
| 14. 5. Do you worry about what will happen to you | List: PEDSQL2 <input type="text" value="6"/> |

SOCIAL FUNCTIONING (problems with...)

- | | |
|--|--|
| 15. 1. Is it hard for you to get along with other kids | List: PEDSQL2 <input type="text" value="6"/> |
| 16. 2. Do other kids say they do not want to play with you | List: PEDSQL2 <input type="text" value="6"/> |
| 17. 3. Do other kids tease you | List: PEDSQL2 <input type="text" value="6"/> |
| 18. 4. Can other kids do things you cannot do | List: PEDSQL2 <input type="text" value="6"/> |
| 19. 5. Is it hard for you to keep up when you play with other kids | List: PEDSQL2 <input type="text" value="6"/> |

SCHOOL FUNCTIONING (problems with...)

- | | |
|--|--|
| 20. 1. Is it hard for you to pay attention in school | List: PEDSQL2 <input type="text" value="6"/> |
| 21. 2. Do you forget things | List: PEDSQL2 <input type="text" value="6"/> |
| 22. 3. Is it hard to keep up with schoolwork | List: PEDSQL2 <input type="text" value="6"/> |
| 23. 4. Do you miss school because of not feeling good | List: PEDSQL2 <input type="text" value="6"/> |
| 24. 5. Do you miss school because you have to go to the doctor's or hospital | List: PEDSQL2 <input type="text" value="6"/> |

List: PEDSQL2

Label	Value
[Blank]	
Not at all	0
Sometimes	2
A lot	4

Pediatric QOL Inventory CHILD REPORT (QS3F)

In the past **ONE month**, how much of a **problem** has this been for you...

ABOUT MY HEALTH AND ACTIVITIES (problems with...)

1.	1. It is hard for me to walk more than one block	List: PEDSQL1 <input type="text" value="6"/>
2.	2. It is hard for me to run	List: PEDSQL1 <input type="text" value="6"/>
3.	3. It is hard for me to do sports activity or exercise	List: PEDSQL1 <input type="text" value="6"/>
4.	4. It is hard for me to lift something heavy	List: PEDSQL1 <input type="text" value="6"/>
5.	5. It is hard for me to take a bath or shower by myself	List: PEDSQL1 <input type="text" value="6"/>
6.	6. It is hard for me to do chores around the house	List: PEDSQL1 <input type="text" value="6"/>
7.	7. I hurt or ache	List: PEDSQL1 <input type="text" value="6"/>
8.	8. I have low energy	List: PEDSQL1 <input type="text" value="6"/>

ABOUT MY FEELINGS (problems with...)

9.	1. I feel afraid or scared	List: PEDSQL1 <input type="text" value="6"/>
10.	2. I feel sad or blue	List: PEDSQL1 <input type="text" value="6"/>
11.	3. I feel angry	List: PEDSQL1 <input type="text" value="6"/>
12.	4. I have trouble sleeping	List: PEDSQL1 <input type="text" value="6"/>
13.	5. I worry about what will happen to me	List: PEDSQL1 <input type="text" value="6"/>

HOW I GET ALONG WITH OTHERS (problems with...)

14.	1. I have trouble getting along with other kids	List: PEDSQL1 <input type="text" value="6"/>
15.	2. Other kids do not want to be my friend	List: PEDSQL1 <input type="text" value="6"/>
16.	3. Other kids tease me	List: PEDSQL1 <input type="text" value="6"/>
17.	4. I cannot do things that other kids my age can do	List: PEDSQL1 <input type="text" value="6"/>
18.	5. It is hard to keep up when I play with other kids	List: PEDSQL1 <input type="text" value="6"/>

ABOUT SCHOOL (problems with...)

19.	1. It is hard to pay attention in class	List: PEDSQL1 <input type="text" value="6"/>
20.	2. I forget things	List: PEDSQL1 <input type="text" value="6"/>
21.	3. I have trouble keeping up with my schoolwork	List: PEDSQL1 <input type="text" value="6"/>
22.	4. I miss school because of not feeling well	List: PEDSQL1 <input type="text" value="6"/>
23.	5. I miss school to go to the doctor or hospital	List: PEDSQL1 <input type="text" value="6"/>

List:PEDSQL1

Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Pediatric QOL Inventory TEEN REPORT (QS3G)

In the past **ONE month**, how much of a **problem** has this been for you...

ABOUT MY HEALTH AND ACTIVITIES (problems with...)

1.	1. It is hard for me to walk more than one block	List: PEDSQL1 <input type="text" value="6"/>
2.	2. It is hard for me to run	List: PEDSQL1 <input type="text" value="6"/>
3.	3. It is hard for me to do sports activity or exercise	List: PEDSQL1 <input type="text" value="6"/>
4.	4. It is hard for me to lift something heavy	List: PEDSQL1 <input type="text" value="6"/>
5.	5. It is hard for me to take a bath or shower by myself	List: PEDSQL1 <input type="text" value="6"/>
6.	6. It is hard for me to do chores around the house	List: PEDSQL1 <input type="text" value="6"/>
7.	7. I hurt or ache	List: PEDSQL1 <input type="text" value="6"/>
8.	8. I have low energy	List: PEDSQL1 <input type="text" value="6"/>

ABOUT MY FEELINGS (problems with...)

9.	1. I feel afraid or scared	List: PEDSQL1 <input type="text" value="6"/>
10.	2. I feel sad or blue	List: PEDSQL1 <input type="text" value="6"/>
11.	3. I feel angry	List: PEDSQL1 <input type="text" value="6"/>
12.	4. I have trouble sleeping	List: PEDSQL1 <input type="text" value="6"/>
13.	5. I worry about what will happen to me	List: PEDSQL1 <input type="text" value="6"/>

HOW I GET ALONG WITH OTHERS (problems with...)

14.	1. I have trouble getting along with other teens	List: PEDSQL1 <input type="text" value="6"/>
15.	2. Other teens do not want to be my friend	List: PEDSQL1 <input type="text" value="6"/>
16.	3. Other teens tease me	List: PEDSQL1 <input type="text" value="6"/>
17.	4. I cannot do things that other teens my age can do	List: PEDSQL1 <input type="text" value="6"/>
18.	5. It is hard to keep up with my peers	List: PEDSQL1 <input type="text" value="6"/>

ABOUT SCHOOL (problems with...)

19.	1. It is hard to pay attention in class	List: PEDSQL1 <input type="text" value="6"/>
20.	2. I forget things	List: PEDSQL1 <input type="text" value="6"/>
21.	3. I have trouble keeping up with my schoolwork	List: PEDSQL1 <input type="text" value="6"/>
22.	4. I miss school because of not feeling well	List: PEDSQL1 <input type="text" value="6"/>
23.	5. I miss school to go to the doctor or hospital	List: PEDSQL1 <input type="text" value="6"/>

List: PEDSQL1

Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Multidimensional Fatigue Scale PARENT for TODDLERS (QS3H)

In the past **ONE month**, how much of a **problem** has this been for your child...

GENERAL FATIGUE (problems with...)

1.	1. Feeling tired	List: PEDSQL1 <input type="text" value="6"/>
2.	2. Feeling physically weak (not strong)	List: PEDSQL1 <input type="text" value="6"/>
3.	3. Feeling too tired to do things that he/she likes to do	List: PEDSQL1 <input type="text" value="6"/>
4.	4. Feeling too tired to spend time with his/her friends	List: PEDSQL1 <input type="text" value="6"/>
5.	5. Trouble finishing things	List: PEDSQL1 <input type="text" value="6"/>
6.	6. Trouble starting things	List: PEDSQL1 <input type="text" value="6"/>

SLEEP/REST FATIGUE (problems with...)

7.	1. Sleeping a lot	List: PEDSQL1 <input type="text" value="6"/>
8.	2. Difficulty sleeping through the night	List: PEDSQL1 <input type="text" value="6"/>
9.	3. Feeling tired when he/she wakes up in the morning	List: PEDSQL1 <input type="text" value="6"/>
10.	4. Resting a lot	List: PEDSQL1 <input type="text" value="6"/>
11.	5. Taking a lot of naps	List: PEDSQL1 <input type="text" value="6"/>
12.	6. Spending a lot of time in bed	List: PEDSQL1 <input type="text" value="6"/>

COGNITIVE FATIGUE (problems with...)

13.	1. Difficulty keeping his/her attention on things	List: PEDSQL1 <input type="text" value="6"/>
14.	2. Difficulty remembering what people tell him/her	List: PEDSQL1 <input type="text" value="6"/>
15.	3. Difficulty remembering what he/she just heard	List: PEDSQL1 <input type="text" value="6"/>
16.	4. Difficulty thinking quickly	List: PEDSQL1 <input type="text" value="6"/>
17.	5. Trouble remembering what he/she was just thinking	List: PEDSQL1 <input type="text" value="6"/>
18.	6. Trouble remembering more than one thing at a time	List: PEDSQL1 <input type="text" value="6"/>

List: PEDSQL1	
Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Multidimensional Fatigue Scale PARENT (YOUNG CHILD (QS3I))

In the past **ONE month**, how much of a **problem** has this been for your child...

GENERAL FATIGUE (problems with...)

1.	1. Feeling tired	List: PEDSQL1 <input type="text" value="6"/>
2.	2. Feeling physically weak (not strong)	List: PEDSQL1 <input type="text" value="6"/>
3.	3. Feeling too tired to do things that he/she likes to do	List: PEDSQL1 <input type="text" value="6"/>
4.	4. Feeling too tired to spend time with his/her friends	List: PEDSQL1 <input type="text" value="6"/>
5.	5. Trouble finishing things	List: PEDSQL1 <input type="text" value="6"/>
6.	6. Trouble starting things	List: PEDSQL1 <input type="text" value="6"/>

SLEEP/REST FATIGUE (problems with...)

7.	1. Sleeping a lot	List: PEDSQL1 <input type="text" value="6"/>
8.	2. Difficulty sleeping through the night	List: PEDSQL1 <input type="text" value="6"/>
9.	3. Feeling tired when he/she wakes up in the morning	List: PEDSQL1 <input type="text" value="6"/>
10.	4. Resting a lot	List: PEDSQL1 <input type="text" value="6"/>
11.	5. Taking a lot of naps	List: PEDSQL1 <input type="text" value="6"/>
12.	6. Spending a lot of time in bed	List: PEDSQL1 <input type="text" value="6"/>

COGNITIVE FATIGUE (problems with...)

13.	1. Difficulty keeping his/her attention on things	List: PEDSQL1 <input type="text" value="6"/>
14.	2. Difficulty remembering what people tell him/her	List: PEDSQL1 <input type="text" value="6"/>
15.	3. Difficulty remembering what he/she just heard	List: PEDSQL1 <input type="text" value="6"/>
16.	4. Difficulty thinking quickly	List: PEDSQL1 <input type="text" value="6"/>
17.	5. Trouble remembering what he/she was just thinking	List: PEDSQL1 <input type="text" value="6"/>
18.	6. Trouble remembering more than one thing at a time	List: PEDSQL1 <input type="text" value="6"/>

List: PEDSQL1	
Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Multidimensional Fatigue Scale PARENT for CHILDREN (QS3J)

In the past **ONE month**, how much of a **problem** has this been for your child...

GENERAL FATIGUE (problems with...)

1.	1. Feeling tired	List: PEDSQL1 <input type="text" value="6"/>
2.	2. Feeling physically weak (not strong)	List: PEDSQL1 <input type="text" value="6"/>
3.	3. Feeling too tired to do things that he/she likes to do	List: PEDSQL1 <input type="text" value="6"/>
4.	4. Feeling too tired to spend time with his/her friends	List: PEDSQL1 <input type="text" value="6"/>
5.	5. Trouble finishing things	List: PEDSQL1 <input type="text" value="6"/>
6.	6. Trouble starting things	List: PEDSQL1 <input type="text" value="6"/>

SLEEP/REST FATIGUE (problems with...)

7.	1. Sleeping a lot	List: PEDSQL1 <input type="text" value="6"/>
8.	2. Difficulty sleeping through the night	List: PEDSQL1 <input type="text" value="6"/>
9.	3. Feeling tired when he/she wakes up in the morning	List: PEDSQL1 <input type="text" value="6"/>
10.	4. Resting a lot	List: PEDSQL1 <input type="text" value="6"/>
11.	5. Taking a lot of naps	List: PEDSQL1 <input type="text" value="6"/>
12.	6. Spending a lot of time in bed	List: PEDSQL1 <input type="text" value="6"/>

COGNITIVE FATIGUE (problems with...)

13.	1. Difficulty keeping his/her attention on things	List: PEDSQL1 <input type="text" value="6"/>
14.	2. Difficulty remembering what people tell him/her	List: PEDSQL1 <input type="text" value="6"/>
15.	3. Difficulty remembering what he/she just heard	List: PEDSQL1 <input type="text" value="6"/>
16.	4. Difficulty thinking quickly	List: PEDSQL1 <input type="text" value="6"/>
17.	5. Trouble remembering what he/she was just thinking	List: PEDSQL1 <input type="text" value="6"/>
18.	6. Trouble remembering more than one thing at a time	List: PEDSQL1 <input type="text" value="6"/>

List: PEDSQL1	
Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Multidimensional Fatigue Scale PARENT for TEENS (QS3K)

In the past **ONE month**, how much of a **problem** has this been for your child...

GENERAL FATIGUE (problems with...)

1.	1. Feeling tired	List: PEDSQL1 <input type="text" value="6"/>
2.	2. Feeling physically weak (not strong)	List: PEDSQL1 <input type="text" value="6"/>
3.	3. Feeling too tired to do things that he/she likes to do	List: PEDSQL1 <input type="text" value="6"/>
4.	4. Feeling too tired to spend time with his/her friends	List: PEDSQL1 <input type="text" value="6"/>
5.	5. Trouble finishing things	List: PEDSQL1 <input type="text" value="6"/>
6.	6. Trouble starting things	List: PEDSQL1 <input type="text" value="6"/>

SLEEP/REST FATIGUE (problems with...)

7.	1. Sleeping a lot	List: PEDSQL1 <input type="text" value="6"/>
8.	2. Difficulty sleeping through the night	List: PEDSQL1 <input type="text" value="6"/>
9.	3. Feeling tired when he/she wakes up in the morning	List: PEDSQL1 <input type="text" value="6"/>
10.	4. Resting a lot	List: PEDSQL1 <input type="text" value="6"/>
11.	5. Taking a lot of naps	List: PEDSQL1 <input type="text" value="6"/>
12.	6. Spending a lot of time in bed	List: PEDSQL1 <input type="text" value="6"/>

COGNITIVE FATIGUE (problems with...)

13.	1. Difficulty keeping his/her attention on things	List: PEDSQL1 <input type="text" value="6"/>
14.	2. Difficulty remembering what people tell him/her	List: PEDSQL1 <input type="text" value="6"/>
15.	3. Difficulty remembering what he/she just heard	List: PEDSQL1 <input type="text" value="6"/>
16.	4. Difficulty thinking quickly	List: PEDSQL1 <input type="text" value="6"/>
17.	5. Trouble remembering what he/she was just thinking	List: PEDSQL1 <input type="text" value="6"/>
18.	6. Trouble remembering more than one thing at a time	List: PEDSQL1 <input type="text" value="6"/>

List: PEDSQL1	
Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Multidimensional Fatigue Scale YOUNG CHILD (QS3L)

Think about how you have been doing for the past few weeks. Please listen carefully to each sentence and tell me how much of a problem this is for you.

After reading the item, gesture to the template. If the child hesitates or does not seem to understand how to answer, read the response options while pointing at the faces.

General Fatigue (PROBLEMS WITH...)

1.	1. Do you feel tired	List: PEDSQL2 <input type="text" value="6"/>
2.	2. Do you feel physically weak (not strong)	List: PEDSQL2 <input type="text" value="6"/>
3.	3. Do you feel too tired to do things that you like to do	List: PEDSQL2 <input type="text" value="6"/>
4.	4. Do you feel too tired to spend time with your friends	List: PEDSQL2 <input type="text" value="6"/>
5.	5. Do you have trouble finishing things	List: PEDSQL2 <input type="text" value="6"/>
6.	6. Do you have trouble starting things	List: PEDSQL2 <input type="text" value="6"/>

Remember, tell me how much of a problem this has been for you for the last few weeks.

Sleep/Rest Fatigue (PROBLEMS WITH...)

7.	1. Do you sleep a lot	List: PEDSQL2 <input type="text" value="6"/>
8.	2. Is it hard for you to sleep through the night	List: PEDSQL2 <input type="text" value="6"/>
9.	3. Do you feel tired when you wake up in the morning	List: PEDSQL2 <input type="text" value="6"/>
10.	4. Do you rest a lot	List: PEDSQL2 <input type="text" value="6"/>
11.	5. Do you take a lot of naps	List: PEDSQL2 <input type="text" value="6"/>
12.	6. Do you spend a lot of time in bed	List: PEDSQL2 <input type="text" value="6"/>

Cognitive Fatigue (PROBLEMS WITH...)

13.	1. Is it hard for you to keep your attention on things	List: PEDSQL2 <input type="text" value="6"/>
14.	2. Is it hard for you to remember what people tell you	List: PEDSQL2 <input type="text" value="6"/>
15.	3. Is it hard for you to remember what you just heard	List: PEDSQL2 <input type="text" value="6"/>
16.	4. Is it hard for you to think quickly	List: PEDSQL2 <input type="text" value="6"/>
17.	5. Do you have trouble remembering what you were just thinking	List: PEDSQL2 <input type="text" value="6"/>
18.	6. Do you have trouble remembering more than one thing at a time	List: PEDSQL2 <input type="text" value="6"/>

List: PEDSQL2

Label	Value
[Blank]	
Not at all	0
Sometimes	2
A lot	4

Multidimensional Fatigue Scale CHILD (QS3M)

In the past **ONE month**, how much of a **problem** has this been for you

General Fatigue (PROBLEMS WITH...)

1.	1. I feel tired	List: PEDSQL1 <input type="text" value="6"/>
2.	2. I feel physically weak (not strong)	List: PEDSQL1 <input type="text" value="6"/>
3.	3. I feel too tired to do things that I like to do	List: PEDSQL1 <input type="text" value="6"/>
4.	4. I feel too tired to spend time with my friends	List: PEDSQL1 <input type="text" value="6"/>
5.	5. I have trouble finishing things	List: PEDSQL1 <input type="text" value="6"/>
6.	6. I have trouble starting things	List: PEDSQL1 <input type="text" value="6"/>

Sleep/Rest Fatigue (PROBLEMS WITH...)

7.	1. I sleep a lot	List: PEDSQL1 <input type="text" value="6"/>
8.	2. It is hard for me to sleep through the night	List: PEDSQL1 <input type="text" value="6"/>
9.	3. I feel tired when I wake up in the morning	List: PEDSQL1 <input type="text" value="6"/>
10.	4. I rest a lot	List: PEDSQL1 <input type="text" value="6"/>
11.	5. I take a lot of naps	List: PEDSQL1 <input type="text" value="6"/>
12.	6. I spend a lot of time in bed	List: PEDSQL1 <input type="text" value="6"/>

Cognitive Fatigue (PROBLEMS WITH...)

13.	1. It is hard for me to keep my attention on things	List: PEDSQL1 <input type="text" value="6"/>
14.	2. It is hard for me to remember what people tell me	List: PEDSQL1 <input type="text" value="6"/>
15.	3. It is hard for me to remember what I just heard	List: PEDSQL1 <input type="text" value="6"/>
16.	4. It is hard for me to think quickly	List: PEDSQL1 <input type="text" value="6"/>
17.	5. I have trouble remembering what I was just thinking	List: PEDSQL1 <input type="text" value="6"/>
18.	6. I have trouble remembering more than one thing at a time	List: PEDSQL1 <input type="text" value="6"/>

List:PEDSQL1

Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Multidimensional Fatigue Scale TEEN (QS3N)

In the past **ONE month**, how much of a **problem** has this been for you

General Fatigue (**PROBLEMS WITH...**)

1.	1. I feel tired	List: PEDSQL1 <input type="text" value="6"/>
2.	2. I feel physically weak (not strong)	List: PEDSQL1 <input type="text" value="6"/>
3.	3. I feel too tired to do things that I like to do	List: PEDSQL1 <input type="text" value="6"/>
4.	4. I feel too tired to spend time with my friends	List: PEDSQL1 <input type="text" value="6"/>
5.	5. I have trouble finishing things	List: PEDSQL1 <input type="text" value="6"/>
6.	6. I have trouble starting things	List: PEDSQL1 <input type="text" value="6"/>

Sleep/Rest Fatigue (**PROBLEMS WITH...**)

7.	1. I sleep a lot	List: PEDSQL1 <input type="text" value="6"/>
8.	2. It is hard for me to sleep through the night	List: PEDSQL1 <input type="text" value="6"/>
9.	3. I feel tired when I wake up in the morning	List: PEDSQL1 <input type="text" value="6"/>
10.	4. I rest a lot	List: PEDSQL1 <input type="text" value="6"/>
11.	5. I take a lot of naps	List: PEDSQL1 <input type="text" value="6"/>
12.	6. I spend a lot of time in bed	List: PEDSQL1 <input type="text" value="6"/>

Cognitive Fatigue (**PROBLEMS WITH...**)

13.	1. It is hard for me to keep my attention on things	List: PEDSQL1 <input type="text" value="6"/>
14.	2. It is hard for me to remember what people tell me	List: PEDSQL1 <input type="text" value="6"/>
15.	3. It is hard for me to remember what I just heard	List: PEDSQL1 <input type="text" value="6"/>
16.	4. It is hard for me to think quickly	List: PEDSQL1 <input type="text" value="6"/>
17.	5. I have trouble remembering what I was just thinking	List: PEDSQL1 <input type="text" value="6"/>
18.	6. I have trouble remembering more than one thing at a time	List: PEDSQL1 <input type="text" value="6"/>

List: PEDSQL1

Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Family Impact Module PARENT REPORT (QS3O)

In the past **ONE month**, as a result of your child's health, how much of a problem have you had with...

PHYSICAL FUNCTIONING (problems with...)

1.	1. I feel tired during the day	List: PEDSQL1 <input type="text" value="6"/>
2.	2. I feel tired when I wake up in the morning	List: PEDSQL1 <input type="text" value="6"/>
3.	3. I feel too tired to do the things I like to do	List: PEDSQL1 <input type="text" value="6"/>
4.	4. I get headaches	List: PEDSQL1 <input type="text" value="6"/>
5.	5. I feel physically weak	List: PEDSQL1 <input type="text" value="6"/>
6.	6. I feel sick to my stomach	List: PEDSQL1 <input type="text" value="6"/>

EMOTIONAL FUNCTIONING (problems with...)

7.	1. I feel anxious	List: PEDSQL1 <input type="text" value="6"/>
8.	2. I feel sad	List: PEDSQL1 <input type="text" value="6"/>
9.	3. I feel angry	List: PEDSQL1 <input type="text" value="6"/>
10.	4. I feel frustrated	List: PEDSQL1 <input type="text" value="6"/>
11.	5. I feel helpless or hopeless	List: PEDSQL1 <input type="text" value="6"/>

SOCIAL FUNCTIONING (problems with...)

12.	1. I feel isolated from others	List: PEDSQL1 <input type="text" value="6"/>
13.	2. I have trouble getting support from others	List: PEDSQL1 <input type="text" value="6"/>
14.	3. It is hard to find time for social activities	List: PEDSQL1 <input type="text" value="6"/>
15.	4. I do not have enough energy for social activities	List: PEDSQL1 <input type="text" value="6"/>

COGNITIVE FUNCTIONING (problems with...)

16.	1. It is hard for me to keep my attention on things	List: PEDSQL1 <input type="text" value="6"/>
17.	2. It is hard for me to remember what people tell me	List: PEDSQL1 <input type="text" value="6"/>
18.	3. It is hard for me to remember what I just heard	List: PEDSQL1 <input type="text" value="6"/>
19.	4. It is hard for me to think quickly	List: PEDSQL1 <input type="text" value="6"/>
20.	5. I have trouble remembering what I was just thinking	List: PEDSQL1 <input type="text" value="6"/>

COMMUNICATION (problems with...)

21.	1. I feel that others do not understand my family's situation	List: PEDSQL1 <input type="text" value="6"/>
22.	2. It is hard for me to talk about my child's health with others	List: PEDSQL1 <input type="text" value="6"/>
23.	3. It is hard for me to tell doctors and nurses how I feel	List: PEDSQL1 <input type="text" value="6"/>

In the past **ONE month**, as a result of your child's health, how much of a problem have **you** had with...

WORRY (problems with...)

24.	1. I worry about whether or not my child's medical treatments are working	List: PEDSQL1 <input type="text" value="6"/>
25.	2. I worry about the side effects of my child's medications/medical treatments	List: PEDSQL1 <input type="text" value="6"/>

26.	3. I worry about how others will react to my child's condition	List: PEDSQL1 <input type="text" value="6"/>
27.	4. I worry about how my child's illness is affecting other family members	List: PEDSQL1 <input type="text" value="6"/>
28.	5. I worry about my child's future	List: PEDSQL1 <input type="text" value="6"/>

DIRECTIONS

Below is a list of things that might be a problem for **your family**. Please tell us **how much of a problem** each one has been for **your family** during the **past ONE month**.

*In the past **ONE month**, as a result of your child's health, how much of a problem has **your family** had with...*

DAILY ACTIVITIES (problems with...)

29.	1. Family activities taking more time and effort	List: PEDSQL1 <input type="text" value="6"/>
30.	2. Difficulty finding time to finish household tasks	List: PEDSQL1 <input type="text" value="6"/>
31.	3. Feeling too tired to finish household tasks	List: PEDSQL1 <input type="text" value="6"/>

FAMILY RELATIONSHIPS (problems with...)

32.	1. Lack of communication between family members	List: PEDSQL1 <input type="text" value="6"/>
33.	2. Conflicts between family members	List: PEDSQL1 <input type="text" value="6"/>
34.	3. Difficulty making decisions together as a family	List: PEDSQL1 <input type="text" value="6"/>
35.	4. Difficulty solving family problems together	List: PEDSQL1 <input type="text" value="6"/>
36.	5. Stress or tension between family members	List: PEDSQL1 <input type="text" value="6"/>

List:PEDSQL1

Label	Value
[Blank]	
Never	0
Almost Never	1
Sometimes	2
Often	3
Almost Always	4

Xanthoma Scale (QS7)

1. Was Xanthoma Scale completed?

List: YES_NO 6

**2. Date of assessment

 dd-mm-yyyy

**3. Clinician Xanthoma Scale score

List: Xanthoma 6

** Conditional Question

List: YES_NO		List: Xanthoma	
Label	Value	Label	Value
[Blank]		[Blank]	
Yes	Y	0-None	0
No	N	1-Minimal	1
		2-Moderate	2
		3-Disfiguring	3
		4-Disabling	4

Caregiver Impression of Change (QS5)

1. Was caregiver impression of change completed?

List: YES_NO 6

**2. Date of assessment

dd-mm-yyyy

**3. How would you rate the change in your child's xanthoma severity since the start of the study?

List: PIC 6

** Conditional Question

List: YES_NO		List: PIC	
Label	Value	Label	Value
[Blank]		[Blank]	
Yes	Y	Much better	1
No	N	Better	2
		A little better	3
		No change	4
		A little worse	5
		Worse	6
		Much worse	7

Subject Summary (DS)

1. Did the subject complete study treatment?	List: YES_NO 6
**2. Date of Discontinuation	<input type="text"/> dd-mm-yyyy
**3. Reason for Discontinuation	List: NCOMPLT 6
4. Date of last dose	<input type="text"/> dd-mm-yyyy
5. Date of final study contact	<input type="text"/> dd-mm-yyyy
**6. Reason for screen failure	List: SF 6
**7. Specify	<input type="text"/>

** Conditional Question

List: YES_NO		List: NCOMPLT		List: SF	
Label	Value	Label	Value	Label	Value
[Blank]		[Blank]		[Blank]	
Yes	Y	Adverse Event	1	Failed to meet eligibility criteria	1
No	N	Death	2	Lost to follow-up	2
		Lost to follow-up	4	Withdrawal by caregiver	3
		Non-compliance with study drug	5	Withdrawal by subject	4
		Physician decision	6		
		Pregnancy	7		
		Progressive Disease	8		
		Protocol violation	9		
		Study terminated by sponsor	11		
		Withdrawal by caregiver	12		
		Withdrawal by subject	13		
		Roll-over to new LUM001/SHP625 study	14		
		Screen Failure	15		
		Other	99		

Protocol Extension (PRE)

1. Did the subject consent to the Protocol Extension Amendment? List: Y_N_NA_ 6
- **2. Protocol Criteria Version List: IEPRT2_ 6
- **3. Date of Consent dd-mm-yyyy
- **4. Did the subject have a > 7 day gap in study dosing? List: YES_NO 6
- **5. Did the subject meet all eligibility criteria? List: YES_NO 6
- ** Conditional Question

List:Y_N_NA_		List:IEPRT2_		List:YES_NO	
Label	Value	Label	Value	Label	Value
[Blank]		[Blank]		[Blank]	
Yes	Y	Amendment	Amendment	Yes	Y
No	N	4 -	4 -	No	N
Not Approached	NA_	27APR2016	27APR2016		

Inclusion/Exclusion Criteria 2 (IE2)

1. Did the subject meet all eligibility criteria?

List: YES_NO 6

2. Inclusion/Exclusion Criteria

2.R.	Criterion Type	Criterion not met	Specify	Approval date	Sponsor approval?
2.R.1.	Criterion Type				List: IE 6
2.R.2.	Criterion not met				List: IENUM 6
2.R.3.	Specify				
**2.R.4.	Date of approval				
2.R.5.	Lumena approval received?				List: YES_NO 6

dd-mm-yyyy

Add New Row

** Conditional Question

List: YES_NO		List: IE		List: IENUM	
Label	Value	Label	Value	Label	Value
[Blank]		[Blank]		[Blank]	
Yes	Y	Inclusion	1	1	1
No	N	Exclusion	2	2	2
				3	3
				4	4
				5	5
				6	6
				7	7
				8	8
				9	9
				10	10

Medical History 2 (MH2)

1. Any new medical histories to report?

List: YES_NO 6

Please only record events that started within a study gap period. A study gap period occurs between completing the prior core or follow up period and consenting to an additional followup period. However, all events that started within 30 days of last dose must be recorded on the Adverse Event form.

2. Medical History

2.R.	Verbatim Term	Start Date (Day)	Start Date (Month)	Start Date (Year)	Ongoing
	End Date (Day)	End Date (Month)	End Date (Year)		
2.R.1.	Verbatim term				
*2.R.1.1.	MHTERM_Enc_TERM				Read Only: Encoded
*2.R.1.2.	MHTERM_Enc_CODE				Read Only: Encoded
*2.R.1.3.	MHTERM_Enc_TYPE				Read Only: Encoded
*2.R.1.4.	MHTERM_Enc_CAT1				Read Only: Encoded
*2.R.1.5.	MHTERM_Enc_CAT2				Read Only: Encoded
*2.R.1.6.	MHTERM_Enc_CAT3				Read Only: Encoded
*2.R.1.7.	MHTERM_Enc_CAT4				Read Only: Encoded
*2.R.1.8.	MHTERM_Enc_CAT5				Read Only: Encoded
*2.R.1.9.	MHTERM_Enc_CAT6				Read Only: Encoded
*2.R.1.10.	MHTERM_Enc_CAT7				Read Only: Encoded
*2.R.1.11.	MHTERM_Enc_CAT8				Read Only: Encoded
*2.R.1.12.	MHTERM_Enc_CAT9				Read Only: Encoded
2.R.2.	Start Date (Day)				List: DAY 6
2.R.3.	Start Date (Month)				List: MONTH 6
2.R.4.	Start Date (Year)				
*2.R.4.1.	Start Date				Read Only: Derived
2.R.5.	Ongoing?				List: YES 6
**2.R.6.	End Date (Day)				List: DAY 6
**2.R.7.	End Date (Month)				List: MONTH 6
**2.R.8.	End Date (Year)				
*2.R.8.1.	End Date				Read Only: Derived

Add New Row

* Hidden Question

** Conditional Question

List: YES_NO		List: DAY		List: MONTH	
Label	Value	Label	Value	Label	Value
[Blank]		[Blank]		[Blank]	
Yes	Y	01	01	JAN	JAN
No	N	02	02	FEB	FEB
		03	03	MAR	MAR
		04	04	APR	APR
		05	05	MAY	MAY

06	06	JUN	JUN
07	07	JUL	JUL
08	08	AUG	AUG
09	09	SEP	SEP
10	10	OCT	OCT
11	11	NOV	NOV
12	12	DEC	DEC
13	13	UNK	UNK
14	14		
15	15		
16	16		
17	17		
18	18		
19	19		
20	20		
21	21		
22	22		
23	23		
24	24		
25	25		
26	26		
27	27		
28	28		
29	29		
30	30		
31	31		
UN	UN		

List: YES	
Label	Value
[Blank]	
YES	Y

Clinical Laboratory Tests (LB)




1. Clinical Laboratory Tests

1.R.	Sample Type	Sample collected?	Collection Date	Sample ID
1.1.1.	Sample Type			Blood
1.1.2.	Was the sample collected?			List: YES_NO 6
**1.1.3.	Collection Date			<input type="text"/> dd-mmm-yyyy
**1.1.4.	Sample ID			<input type="text"/>
1.2.1.	Sample Type			Urine
1.2.2.	Was the sample collected?			List: YES_NO 6
**1.2.3.	Collection Date			<input type="text"/> dd-mmm-yyyy
**1.2.4.	Sample ID			<input type="text"/>

** Conditional Question

List: YES_NO	
Label	Value
[Blank]	
Yes	Y
No	N

Long Term Optional Treatment (PRE2)

1.	Did the subject consent to the long term optional treatment period?	List: YES_NO <input type="text" value="6"/>
**2.	Protocol Criteria Version	List: IEPRT3_ <input type="text" value="6"/>
**3.	Date Parental Informed Consent signed	<input type="text"/>  dd-mmm-yyyy
**4.	Young Adult Consent Not Applicable	List: NA <input type="text" value="6"/>
**5.	Date Young Adult Consent signed	<input type="text"/>  dd-mmm-yyyy
**6.	Assent Not Applicable	List: NA <input type="text" value="6"/>
**7.	Date Assent signed	<input type="text"/>  dd-mmm-yyyy
**8.	What schedule did subject complete first?	List: PROD <input type="text" value="6"/>

** Conditional Question

List: YES_NO	
Label	Value
[Blank]	
Yes	Y
No	N

List: IEPRT3_	
Label	Value
[Blank]	
Amendment 5 - 13NOV2017	Amendment 5 - 13NOV2017
Amendment 6 - 25-JUN-2018	Amendment 6 - 25-JUN-2018

List: NA	
Label	Value
[Blank]	
Not Applicable	NA

List: PROD	
Label	Value
[Blank]	
D	D
E	E
Not Applicable - Screen Failure	NA

Inclusion/Exclusion Criteria 3 (IE3)

1. Did the subject meet all eligibility criteria?

List: YES_NO 6

2. Inclusion/Exclusion Criteria

2.R. Criterion Type

Criterion not met

2.R.1. Criterion Type

List: IE 6

2.R.2. Criterion not met

List: IENUM2_ 6

[Add New Row](#)

List:YES_NO		List:IE		List:IENUM2_	
Label	Value	Label	Value	Label	Value
[Blank]		[Blank]		[Blank]	
Yes	Y	Inclusion	1	1	1
No	N	Exclusion	2	2	2
				3	3
				4	4
				5	5
				6	6

Subject Summary 2 (DS2)

1. Did the subject complete treatment under long term optional period?

List: YES_NO 6

**2. Date of Discontinuation

 dd-mm-yyyy

**3. Reason for Discontinuation

List: NCOMPLT 6

**4. Specify

5. Date of last dose

 dd-mm-yyyy

6. Date of final study contact

 dd-mm-yyyy

** Conditional Question

List: YES_NO		List: NCOMPLT	
Label	Value	Label	Value
[Blank]		[Blank]	
Yes	Y	Adverse Event	1
No	N	Death	2
		Lost to follow-up	4
		Non-compliance with study drug	5
		Physician decision	6
		Pregnancy	7
		Progressive Disease	8
		Protocol violation	9
		Study terminated by sponsor	11
		Withdrawal by caregiver	12
		Withdrawal by subject	13
		Roll-over to new LUM001/SHP625 study	14
		Screen Failure	15
		Other	99

Long Term Optional Treatment 2 (PRE3)

1. Did the subject consent to the long term optional treatment 2 period for Protocol Amendment 6?

List: YES_NO 6

- **2. Date Parental Informed Consent signed

dd-mmm-yyyy

- **3. Young Adult Consent Not Applicable

List: NA 6

- **4. Date Young Adult Consent signed

dd-mmm-yyyy

- **5. Assent Not Applicable

List: NA 6

- **6. Date Assent signed

dd-mmm-yyyy

** Conditional Question

List: YES_NO		List: NA	
Label	Value	Label	Value
[Blank]		[Blank]	
Yes	Y	Not	NA
No	N	Applicable	

Concomitant Medications (CM)

1. Were any medications taken?

List: YES_NO 6

2. Concomitant Medications

Medication Name	Reported in core (301)?	Indication	Start Date (Day)	Start Date (Month)
Start Date (Year)	Taken Prior to Day 0 of 305 Study?	Ongoing at Week 48?	Prior to entering the Amend #4?	Ongoing?
End Date (Day)	End Date (Month)	End Date (Year)	Dose	Dose Unit
Route	Frequency			

2.R.1. Medication/Therapy Name

*2.R.1.1. CMTRT_Enc_TERM

Read Only: Encoded

*2.R.1.2. CMTRT_Enc_CODE

Read Only: Encoded

*2.R.1.3. CMTRT_Enc_TYPE

Read Only: Encoded

*2.R.1.4. CMTRT_Enc_CAT1

Read Only: Encoded

*2.R.1.5. CMTRT_Enc_CAT2

Read Only: Encoded

*2.R.1.6. CMTRT_Enc_CAT3

Read Only: Encoded

*2.R.1.7. CMTRT_Enc_CAT4

Read Only: Encoded

*2.R.1.8. CMTRT_Enc_CAT5

Read Only: Encoded

*2.R.1.9. CMTRT_Enc_CAT6

Read Only: Encoded

*2.R.1.10. CMTRT_Enc_CAT7

Read Only: Encoded

*2.R.1.11. CMTRT_Enc_CAT8

Read Only: Encoded

*2.R.1.12. CMTRT_Enc_CAT9

Read Only: Encoded

2.R.2. Reported in core (301) study database?

List: YES_NO 6

2.R.3. Indication

2.R.4. Start Date (Day)

List: DAY 6

2.R.5. Start Date (Month)

List: MONTH 6

2.R.6. Start Date (Year)

*2.R.6.1. Start Date

Read Only: Derived

2.R.7. Taken Prior to Day 0 of 305 Study?

List: YES_NO 6

2.R.8. Was this medication ongoing at Week 48 of 305 study?

List: YES_NO 6

2.R.9. Taken Prior to entering the Amendment #4 305 Extension?

List: YES_NO 6

2.R.10. Ongoing at subject final visit?

List: YES_NO 6

**2.R.11. End Date (Day)

List: DAY 6

**2.R.12. End Date (Month)

List: MONTH 6

**2.R.13. End Date (Year)

*2.R.13.1. End Date

Read Only: Derived

2.R.14. Dose

2.R.15. Dose Unit

List: DOSE 6

**2.R.16. Other: Specify

2.R.17. Route

List: ROUTE 6

**2.R.18. Other: Specify

2.R.19. Frequency

List: FREQ 6

**2.R.20. Other: Specify

[Add New Row](#)

* Hidden Question

** Conditional Question

List: YES_NO

Label	Value
[Blank]	
Yes	Y
No	N

List: DAY

Label	Value
[Blank]	
01	01
02	02
03	03
04	04
05	05
06	06
07	07
08	08
09	09
10	10
11	11
12	12
13	13
14	14
15	15
16	16
17	17
18	18
19	19
20	20
21	21
22	22
23	23
24	24
25	25
26	26
27	27
28	28
29	29
30	30
31	31
UN	UN

List: MONTH

Label	Value
[Blank]	
JAN	JAN
FEB	FEB
MAR	MAR
APR	APR
MAY	MAY
JUN	JUN
JUL	JUL
AUG	AUG
SEP	SEP
OCT	OCT
NOV	NOV
DEC	DEC
UNK	UNK

List: DOSE

List: ROUTE

List: FREQ

Label	Value	Label	Value	Label	Value
[Blank]		[Blank]		[Blank]	
TABLET	TABLET	ORAL	ORAL	BID	BID
CAPSULE	CAPSULE	INTRAVENOUS	INTRAVENOUS	ONCE	ONCE
mg	mg	SUBCUTANEOUS	SUBCUTANEOUS	PRN	PRN
ug	ug	TOPICAL	TOPICAL	Q6H	Q6H
g	g	TRANSDERMAL	TRANSDERMAL	Q8H	Q8H
mEq	mEq	RESPIRATORY (INHALATION)	RESPIRATORY (INHALATION)	Q12H	Q12H
mL	mL	NASAL	NASAL	Q3D	Q3D
oz	oz	INTRASINAL	INTRASINAL	QD	QD
L/min	L/min	OPHTHALMIC	OPHTHALMIC	QID	QID
gtt	gtt	INTRAMUSCULAR	INTRAMUSCULAR	QOD	QOD
IU	IU	VAGINAL	VAGINAL	QM	QM
U	U	RECTAL	RECTAL	QS	QS
PATCH	PATCH	SUBLINGUAL	SUBLINGUAL	TID	TID
PUFF	PUFF	BUCCAL	BUCCAL	TIS	TIS
SPRAY	SPRAY	INTRA-ARTICULAR	INTRA-ARTICULAR	UNKNOWN	UNKNOWN
Tbsp	Tbsp	AURICULAR (OTIC)	AURICULAR (OTIC)	Other	Other
tsp	tsp	UNKNOWN	UNKNOWN		
UNKNOWN	UNKNOWN	OTHER	OTHER		
OTHER	OTHER				

Study Drug Administration 2 (EX2)

1. Dose Escalation Period

1.R.	Start Date	End Date	Dose
1.R.1.	Start Date	<input type="text"/>	<input type="text" value="dd-mmm-yyyy"/>
1.R.2.	End Date	<input type="text"/>	<input type="text" value="dd-mmm-yyyy"/>
1.R.3.	Dose	List: EXDOSE <input type="text" value="6"/>	
<input type="button" value="Add New Row"/>			

2. Stable Dosing and Optional Extension

2.R.	Start Date	End Date	Dose
2.R.1.	Start Date	<input type="text"/>	<input type="text" value="dd-mmm-yyyy"/>
2.R.2.	End Date	<input type="text"/>	<input type="text" value="dd-mmm-yyyy"/>
2.R.3.	Dose	List: EXDOSEB <input type="text" value="6"/>	
<input type="button" value="Add New Row"/>			

List:EXDOSE		List:EXDOSEB	
Label	Value	Label	Value
[Blank]		[Blank]	
14ug/kg/day or LUM001- 301 Dose	14	0 ug/kg/day	0
35 ug/kg/day or LUM001- 301 Dose	35	14 ug/kg/day	14
70 ug/kg/day or LUM001- 301 Dose	70	35 ug/kg/day	35
140 ug/kg/day or LUM001- 301 Dose	140	70 ug/kg/day	70
280 ug/kg/day or LUM001- 301 Dose	280	140 ug/kg/day	140
		280 ug/kg/day	280

Adverse Events (AE)

1. Were any Adverse Events experienced?

List: YES_NO 6

2. Adverse Events

Verbatim Term	Reported in core (301)?	Serious?	Start Date (Day)	Start Date (Month)
Start Date (Year)	Outcome	End Date (Day)	End Date (Month)	End Date (Year)
Ongoing at Week 48?	Action Taken	Relationship	Severity	Life Threatening
Treatment: None	Treatment: Medication	Treatment: Hospitalization	Other: Specify	

2.R.1. Verbatim Term

*2.R.1.1. AETERM_Enc_TERM

Read Only: Encoded

*2.R.1.2. AETERM_Enc_CODE

Read Only: Encoded

*2.R.1.3. AETERM_Enc_TYPE

Read Only: Encoded

*2.R.1.4. AETERM_Enc_CAT1

Read Only: Encoded

*2.R.1.5. AETERM_Enc_CAT2

Read Only: Encoded

*2.R.1.6. AETERM_Enc_CAT3

Read Only: Encoded

*2.R.1.7. AETERM_Enc_CAT4

Read Only: Encoded

*2.R.1.8. AETERM_Enc_CAT5

Read Only: Encoded

*2.R.1.9. AETERM_Enc_CAT6

Read Only: Encoded

*2.R.1.10. AETERM_Enc_CAT7

Read Only: Encoded

*2.R.1.11. AETERM_Enc_CAT8

Read Only: Encoded

*2.R.1.12. AETERM_Enc_CAT9

Read Only: Encoded

2.R.2. Reported in core (301) study database?

List: YES_NO 6

2.R.3. Is the Adverse Event Serious?

List: YES_NO 6

2.R.4. Start Date (Day)

List: DAY 6

2.R.5. Start Date (Month)

List: MONTH 6

2.R.6. Start Date (Year)

*2.R.6.1. Start Date

Read Only: Derived

2.R.7. Outcome

List: OUT 6

**2.R.8. End Date (Day)

List: DAY 6

**2.R.9. End Date (Month)

List: MONTH 6

**2.R.10. End Date (Year)

*2.R.10.1. End Date

Read Only: Derived

2.R.11. Was this event ongoing at Week 48 of 305 study?

List: YES_NO 6

2.R.12. Action Taken with Study Treatment

List: AEACN 6

2.R.13. Relationship to Study Treatment

List: REL 6

2.R.14. Was CTCAE grading used to assess severity?

List: YES_NO 6

2.R.15. Severity

List: SEV 6

**2.R.16. Was the Serious Event Life Threatening?

List: YES_NO 6

2.R.17. Treatment Required: None

List: YES 6

**2.R.18. Treatment Required: Medication Required

List: YES 6

**2.R.19. Treatment Required: Hospitalization Required

List: YES 6

**2.R.20. Treatment Required: Other

List: YES 6

**2.R.21. Other: Specify

[Add New Row](#)

* Hidden Question

** Conditional Question

List: YES_NO	
Label	Value
[Blank]	
Yes	Y
No	N

List: DAY	
Label	Value
[Blank]	
01	01
02	02
03	03
04	04
05	05
06	06
07	07
08	08
09	09
10	10
11	11
12	12
13	13
14	14
15	15
16	16
17	17
18	18
19	19
20	20
21	21
22	22
23	23
24	24
25	25
26	26
27	27
28	28
29	29
30	30
31	31
UN	UN

List: MONTH	
Label	Value
[Blank]	
JAN	JAN
FEB	FEB
MAR	MAR
APR	APR
MAY	MAY
JUN	JUN
JUL	JUL
AUG	AUG
SEP	SEP
OCT	OCT
NOV	NOV
DEC	DEC
UNK	UNK

List:OUT	
Label	Value
[Blank]	
Fatal	1
Not Recovered/not resolved	2
Recovered/Resolved	3
Recovered/Resolved with sequelae	4
Recovering/Resolving	5
Unknown	88

List:AEACN	
Label	Value
[Blank]	
Dose not changed	1
Drug interrupted	2
Drug withdrawn	3
Dose reduced	4
Not Applicable	5


List:REL	
Label	Value
[Blank]	
1 - Related	1
2 - Possibly Related	2
3 - Unlikely/Remotely Related	3
4 - Not Related	4

List:SEV	
Label	Value
[Blank]	
Grade 1: Mild	1
Grade 2: Moderate	2
Grade 3: Severe	3
Grade 4: Life-threatening	4
Grade 5: Fatal	5


List:YES	
Label	Value
[Blank]	
YES	Y

Study Drug Accountability 1 (SDA1)

1. Study Drug Accountability

1.R.	Unique Batch	Vial Weeks	Batch Vial Number	Dispensed Date
1.R.1.	Unique Batch- 117462/C/xx			
1.R.2.	Vial Weeks (xx) or (xx-xx)			
1.R.3.	Batch Vial Number		Read Only: Derived	
1.R.4.	Dispensed Date			 dd-mmm-yyyy
Add New Row				

Screened (Scr)

1. Subject ID:
2. Subject Initials:
3. Screen Date:  dd-mm-yyyy

Enrolled (Enroll)

1. Subject ID:

2. Enrolled Date:



dd-mmm-yyyy

Failed (Fail)

1. Failed Date:

 dd-mm-yyyy

Discontinued (Disc)

1. Discontinued Date:

 dd-mmm-yyyy

Completed (Comp)

1. Completed Date:

 dd-mmm-yyyy