INSTRUCTIONS: Complete this form for all subjects who have an A103 form expected in ADEPT.

Section A: KEY IDENTIFYING INFORMATION

A1. Study Identification Number ___ ___ - ___ ___ ___ ___ ___ ___ - ___

A2. Study visit BASELINE.....(0)

a. What number screening is this? ___

A3. Date of form completion ___ ___ / ___ ___ / ___ ___ ___ ___
M M D D Y Y Y Y

A4. Name of person completing form _____ ___ ___ ___
PRINT FULL NAME INITIALS

A5. Date of visit ___ ___ / ___ ___ / ___ ___ ___ ___
M M D D Y Y Y Y

Section B: GHENT CRITERIA

Those items listed in **boldface** are major manifestations that contribute to major criteria or system involvement. Those items NOT in boldface are minor manifestations that contribute only to system involvement. The data reported in this section will be used to determine the subject's eligibility. Use the data from the ADEPT A100 Report and the ADEPT Calculator to answer the questions below.

B1. Skeletal System (for B1c refer to the ADEPT Calculator for ratios)	**YES**	**NO**	**UNKNOWN**
a. Pectus carinatum	**1**	**2**	**-8**
b. Pectus excavatum (subjectively judged as moderate to severe)	**1**	**2**	**-8**
c. Reduced upper-to-lower segment ratio for age OR arm span-to-height ratio > 1.05	**1**	**2**	**-8**
d. Wrist AND thumb signs	**1**	**2**	**-8**
e. Scoliosis > 20° OR spondylolisthesis	**1**	**2**	**-8**
f. Reduced extension at the elbow (< 170°)	**1**	**2**	**-8**
g. Medial rotation of the medial malleolus causing pes planus	**1**	**2**	**-8**
h. Protrusio acetabuli of any degree (ascertained on radiographs)	**1**	**2**	**-8**
i. Mild* pectus excavatum (*Modified slightly from published criteria)	1	2	-8
j. Joint hypermobility	1	2	-8
k. Highly arched palate	1	2	-8
l. Facial (If any of the following are present, circle "YES")	1	2	-8

- Dolichocephaly
- Malar hypoplasia
- Enophthalmos
- Retrognathia
- Down-slanting palpebral fissures

		YES	NO	UNKNOWN
B2.	**Ocular System**			
	a. Ectopia lentis	**1**	**2**	**-8**
	b. Flat cornea	1	2	-8
	c. Increased axial length of the globe	1	2	-8
	d. Hypoplastic iris **OR** hypoplastic ciliary muscle causing decreased miosis	1	2	-8

		YES	NO	UNKNOWN
B3.	**Cardiovascular System (for B3b refer to ADEPT A100 report that provides z-score calculations)**			
	a. Mitral valve prolapse	1	2	-8
	b. Dilatation of the main pulmonary artery (Z-score > 2.0) in the absence of valvular or peripheral stenosis	1	2	-8
	c. Calcification of the mitral annulus	1	2	-8
	d. Dilatation of the descending aorta	1	2	-8

		YES	NO	UNKNOWN
B4.	**Pulmonary System**			
	a. Spontaneous pneumothorax	1	2	-8
	b. Apical blebs (by chest X-ray, CT or MRI)	1	2	-8

		YES	NO	UNKNOWN
B5.	**Skin and Integument**			
	a. Striae distensae	1	2	-8
	b. Recurrent or incisional hernia	1	2	-8

		YES	NO	UNKNOWN
B6.	**Dura**			
	a. Lumbosacral dural ectasia (by CT or MRI)	**1**	**2**	**-8**

		YES	NO	UNKNOWN
B7.	**Family and genetic history**			
	a. First degree relative who independently meets the diagnostic criteria Ghent criteria worksheet should be completed for affected relative	**1**	**2**	**-8**
	b. Presence of a mutation in *FBN1* known to cause Marfan syndrome	**1**	**2**	**-8**
	c. Presence of a haplotype around *FBN1* that is inherited by descent and unequivocally associated with Marfan syndrome in the family	**1**	**2**	**-8**

While the differential diagnosis of Marfan syndrome is extensive, there are only two disorders that can show sufficient manifestations to meet diagnostic criteria for Marfan syndrome; they are Shprintzen-Goldberg syndrome (SGS) and Loeys-Dietz syndrome (LDS). They show a very different natural history and need to be excluded from this trial. Fortunately, both conditions show manifestations that are rarely seen in Marfan syndrome. Observation of any of the following findings will preclude participation, unless an appeal is requested and approved.

B8. Exclusion of other disorders	**YES**	**NO**	**UNKNOWN**
a. Craniosynostosis (SGS and LDS)	1	2	-8
b. Hypertelorism (SGS and LDS)	1	2	-8
c. Mental retardation (SGS and LDS)	1	2	-8
d. Cleft palate (LDS)	1	2	-8
e. Bifid uvula (split or central raphe) (LDS)	1	2	-8
f. Club foot (SGS and LDS)	1	2	-8
g. Documented arterial tortuosity (prominently of neck and head vessels) (LDS)	1	2	-8
h. Chiari malformation (SGS and LDS)	1	2	-8
i. Cervical spine instability (LDS)	1	2	-8

If YES or UNKNOWN to any B8a-f, or YES to any B8g-i, then subject is NOT eligible for the Trial. <u>End Baseline Screening</u>. If you feel the subject should be included in the trial, you may file an appeal. Please document your reason for appeal below (B9a) and include any relevant information not already reported on this form. Then, answer Z1, end form, and wait for appeal request result before proceeding with baseline screening.

B9. Would you like to file an appeal? YES ..... 1 NO..... 2 **(B10)** N/A .....-1 **(B10)**

a. Document your reason for appeal. Include any relevant information not already reported on this form. (After documenting reason, skip question B10 and go to question Z1).

_____

_____

_____

B10. Is the subject eligible to proceed with screening? YES 1

NO 2

Section Z: TIME TO COMPLETE FORM AND SIGNATURE

Z1. How long did it take to complete this form? ___ ___ ___ minutes

Signature of PI: _____ Date: _____

END OF FORM

Please run the "A103" Report in ADEPT to see whether subject satisfies Ghent criteria.