Supplemental Table 4. FACE definitions and outcomes across various Fabry studies

Treatment	Migalastat N=97	Agalsidase beta N=52	Agalsidase beta N=40	Agalsidase alfa or beta N=178	Agalsidase alfa or beta N=387	Agalsidase beta N=1411	Migalastat N=31	Agalsidase beta N=51	Migalastat N=36	Agalsidase beta N=1044
Author, Year	**Current Analysis**	**Germain, 2015**	**Weidemann, 2013**	**Sirrs, 2014**	**Arends, 2018**	**Hopkin, 2016**	**Feldt-Rasmussen, 2020**	**Banikazemi, 2007**	**Hughes, 2017**	**Ortiz, 2016**
Patient population	Pts with amenable mutations (37 M; 60 F)	Adult pts with classic FD (50 M; 2 F)	Adult pts with advanced FD (31 M; 9 F)	Pts with FD meeting Canadian ERT criteria (100 M; 78 F)	Pts with FD (195 M; 192 F)	Pts in the Fabry Registry (969 M; 442 F)	ERT-experienced pts with amenable mutations (16 M; 17 F)*	Adult pts with advanced FD (45 M; 6 F)	ERT-experienced pts with amenable mutations (16 M; 20 F)	Pts in the Fabry Registry (641 M; 403 F)
Length of follow-up, median	5 y	10 y	6 y	5 y	5 y	M: 4 y F: 3 y	2.5 y	1.5 y	1.5 y	NR¶
Events Overall, % of patients	18%	19%	33%	27%	27%	M: 21%; F: 13%	32%	28%	29%	17%
Renal Events, % of patients	2%	7.7%	10%	3%†	3%†	M: 7%; F: 2%	29%	20%	24%	6%
End-stage renal disease	**X**		**X**	**X**	**X**			**X**		
Dialysis	**X**	**X**	**X**		**X**	**X**		**X**		**X**
Transplant	**X**	**X**			**X**	**X**		**X**		**X**

Serum creatinine (predefined increase)	**X**							**X**		
Increased urinary protein (predefined)							**X**		**X**	
GFR decrease (predefined rate)				**X**			**X**		**X**	
Cardiac Events, % of patients	12%	4%	15%	17%	14%	M: 9%; F: 7%	3%	6%	6%	7%
Cardiac-related death						**X**				
Myocardial infarction	**X**	**X**		**X**		**X**	**X**	**X**	**X**	**X**
Chronic heart failure	**X**	**X**		**X**		**X**	**X**	**X**	**X**	**X**
Atrial fibrillation	**X**					**X**				**X**
Ventricular tachy-arrhythmia			**X**	**X**		**X**				**X**
Symptomatic arrhythmia	**X**						**X**	**X**	**X**	

requiring medication or intervention										
Heart disease progressive enough to require pacemaker	**X**	**X**		**X**	**X**	**X**	**X**	**X**	**X**	**X**
Bypass surgery (CABG)		**X**		**X**		**X**				**X**
Coronary artery dilation						**X**				**X**
Implantation of cardioverter or defibrillator	**X**	**X**		**X**	**X**	**X**	**X**	**X**	**X**	**X**
Direct cardioversion	**X**			**X**			**X**	**X**	**X**	
Unstable angina	**X**			**X**			**X**	**X**	**X**	
Percutaneous transluminal coronary angioplasty				**X**						

Valve replacement surgery	**X**			**X**						
Stent	**X**			**X**						
Acute coronary syndrome				**X**						
Heart block				**X**						
Cardiac arrest				**X**						
Cardiac ablation	**X**									
Cerebrovascular Events, % of patients	5%	10%	10%	8%	7%	M: 5%; F: 3%	0%	0%	0%	3%
Stroke	**X**		**X**	**X**	**X**	**X**	**X**	**X**	**X**	**X**
Transient Ischemic Attack	**X**		**X**	**X**	**X**		**X**	**X**	**X**	
Acute hearing loss				**X**						
Death, % of patients	0%		18%	5%	3%	M: 4%; F: 1%	0%	2%	0%	1%
Due to any cause			**X**	**X**	**X**	**X‡**		**X**	**X**	**X**

Due to Fabry disease	**X**						**X**			

CABG, coronary artery bypass grafting; ERT, enzyme replacement therapy; F, females; FACEs, Fabry-associated clinical events; FD, Fabry Disease; GFR, Glomerular Filtration Rate; M, males; NR, not reported; Pts, Patients; y, years.

FACEs are defined as renal, cardiac and cerebrovascular events. The specific renal, cardiac or cerebrovascular events that are included within these definitions for each Fabry study, which are marked by 'X', are provided. *33 patients were originally included in the open-label population; 2 patients were subsequently found to have non-amenable variants by Good Laboratory Practice-validated migalastat amenability assay and were excluded from the efficacy analyses. †Patients needing renal replacement therapy; excludes patients who had end-stage renal disease at study entry. ‡Non-cardiac death. ¶Median follow-up duration was not reported. Maximum follow-up was 5 years.