

Retrospective and Prospective Evaluation of Pediatric Cutaneous Mastocytosis

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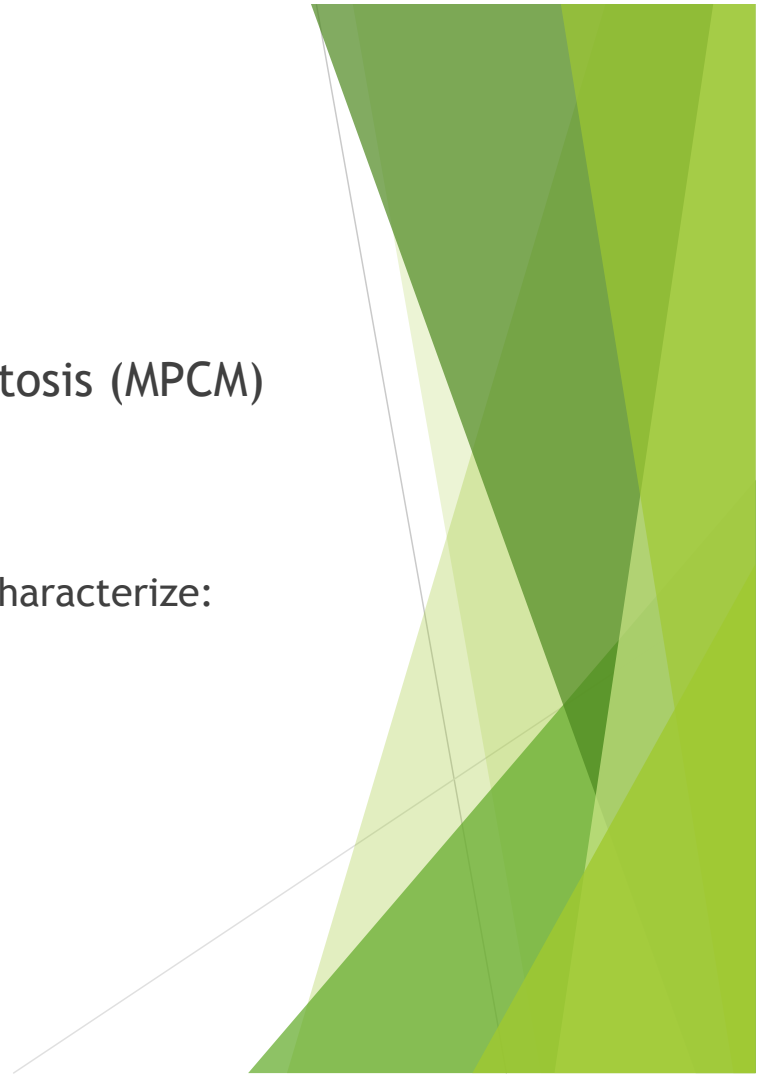
Disclosure statement

No financial relationships relevant to this presentation



Background

- ▶ Registry of Maculopapular Cutaneous Mastocytosis (MPCM) patients
- ▶ Retrospective data (presented here)
- ▶ Prospective data: 20 years clinical data to better characterize:
 - Associated symptoms
 - Markers of severity and/or progression
 - Association with other disorders



Methods

- ▶ Multi-centered study
- ▶ Inclusion criteria:
 - ▶ 5 or more cutaneous mastocytosis lesions
 - ▶ Diagnosed before age 15 (within past 25 yrs)
- ▶ Interview, physical exams to collect information every 1-2 years
- ▶ Data housed in REDCap

Initial analysis

Retrospective data





248 Patients
(403 Entries)

21 patients
with onset >15
years of age

Excluded

227 Patients

Initial analysis

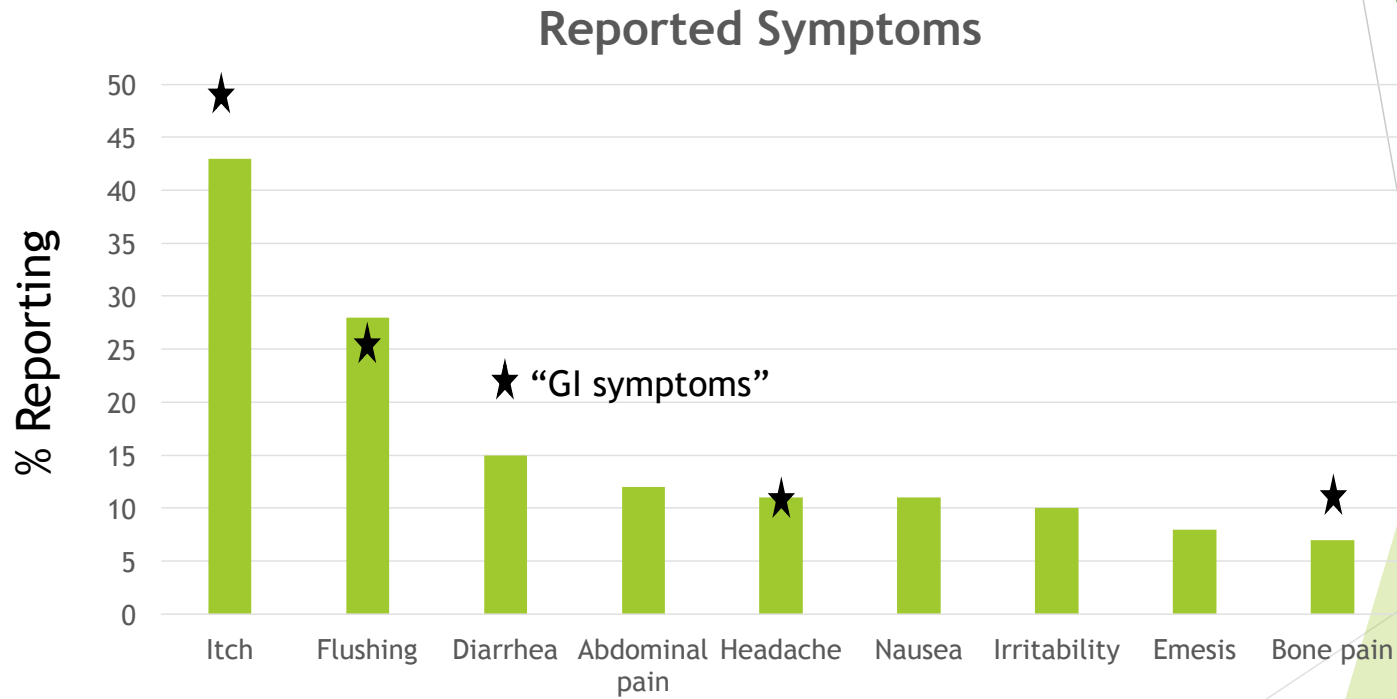
Retrospective data - Demographics

	Present study (n=227)	Meni <i>et al</i> 2015 (n=1747)*	Alvarez-Twose <i>et al</i> 2012 (n=80)
Female: Male ratio	1:1.4	1:1.4	1:0.96
Onset prior to 2 years	94%	91%	NR
Race	78% Caucasian 10% Hispanic 10% other 1% African American	NR	NR
Median age of onset	3 months	NR	3 months

*Meni et al included cases of DCM, TMEP, MCL in addition to MPCM

[Br J Dermatol](#). 2015 Mar;172(3):642-51
[Allergy](#). 2012 Jun;67(6):813-21

Initial Analysis - Symptoms



★ Rate as reported in Meni *et al* 2015, across subtypes

What variables are predictive of number of systemic symptoms?

- ▶ Not significant:
 - ▶ Age of onset (>/< 2 yrs as categorical and continuous variable)
 - ▶ Sex
 - ▶ Race

		Number of systemic symptoms	
Variable	Level	Mean	P-value
Age at visit 1	<=2 years	0.32	<.001
	>2 years	1.18	
Antihistamine	No	0.44	0.028
	Yes	1.19	

Univariate analysis

What variables are predictive of number of systemic symptoms?

		Number of systemic symptoms	
Variable	N	Pearson CC	Pearson P-value
Number of skin symptoms*	222	0.392	<.001
Number macules/ papules	171	0.210	0.006

*Skin symptoms: itching, flushing, bullae, Dariers

Elevated serum tryptase predicts worse mast cell activation symptoms

Alvarez-Twose *et al*: 111 children with mastocytosis (72% MPCM), determined following cutoffs:

- ▶ Need for anti-mediator therapy 6.6ug/l (grade ≥ 2)
- ▶ Hospitalization 15.5 ug/l (grade 4)
- ▶ ICU management 30.8 ug/l
- ▶ Higher in patients with extensive ($>90\%$ BSA) involvement

Serum tryptase is associated with more systemic symptoms

- ▶ Univariate association
- ▶ 77 patients had data for tryptase at visit 1
- ▶ 73 had age of onset and tryptase data

			Number of systemic symptoms	
Serum tryptase	Level (ug/L)	Number	P-value	
	0-<6.6	50	0.58	<.001
6.6-<15.5	19	0.32		
15.5-<30.8	4	0.00		
>30.8	4	3.25		

		Age of onset		
Serum tryptase	Level (ug/L)	<=2 years	>2 years	P-value
	0-<6.6	45 (66.18)	1 (20)	0.019
6.6-<15.5	17 (25)	2 (40)		
15.5-<30.8	4 (5.88)	0 (0)		
>30.8	2 (2.94)	2 (40)		

Are neurocognitive disorders more common in children with mastocytosis?

	Study	Result
Adult	Moura <i>et al</i> 2012	57 patients (mean age 45), 38.6% with cognitive impairment
Pediatric	McFarlin <i>et al</i> 1991	12 children with mastocytosis, no difference in rate of learning difficulties
	Seamens <i>et al</i> 2016	17 pediatric patients completed surveys, 2 with formal diagnosis of cognitive disorder (12%)
	Theoharides 2009	41/400 surveys to mastocytosis society indicating diagnosis of Asperger's or Autism (10%)

[PLoS One](#). 2012;7(6):e39468
[Int J Psychiatry Med](#). 1991;21(3):281-9.
[J Am Acad Dermatol](#). 2016 Dec;75(6):1254-1255
[Int J Immunopathol Pharmacol](#). 2009 Oct-Dec;22(4):859-65.

Initial Analysis - Neurocognitive disorders

- ▶ Fifteen (8.3% CI 4.7-13.4%) patients reported history of neurocognitive disorder
 - ▶ Previously published rate 5.4% (among general population), not significantly different
- ▶ Skin and systemic symptoms did not correlate with neurocognitive disorders (p=0.34 and 0.57)

Next steps

- ▶ Prospective data collection
- ▶ Additional analysis of retrospective data?
 - ▶ Existing longitudinal data



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